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**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**  
Washington, D.C. 20549

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**FORM 6-K**

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**REPORT OF FOREIGN PRIVATE ISSUER  
PURSUANT TO SECTION 13a-16 OR 15d-16  
UNDER THE SECURITIES EXCHANGE ACT OF 1934**

For the month of September, 2024

Commission File Number: 001-36815

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**Ascendis Pharma A/S**

(Exact Name of Registrant as Specified in Its Charter)

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**Tuborg Boulevard 12  
DK-2900 Hellerup  
Denmark**  
(Address of principal executive offices)

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Indicate by check mark whether the registrant files or will file annual reports under cover of Form 20-F or Form 40-F.

Form 20-F  Form 40-F

Indicate by check mark if the registrant is submitting the Form 6-K in paper as permitted by Regulation S-T Rule 101(b)(1):

Indicate by check mark if the registrant is submitting the Form 6-K in paper as permitted by Regulation S-T Rule 101(b)(7):

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## **INCORPORATION BY REFERENCE**

This report on Form 6-K shall be deemed to be incorporated by reference into the registration statements on Form S-8 (Registration Numbers 333-228576, 333-203040, 333-210810, 333-211512, 333-213412, 333-214843, 333-216883, 333-254101, 333-261550, 333-270088 and 333-277519) and Form F-3 (Registration Numbers 333-209336, 333-211511, 333-216882, 333-223134, 333-225284, and 333-256571) of Ascendis Pharma A/S (the “Company”) (including any prospectuses forming a part of such registration statements) and to be a part thereof from the date on which this report is filed, to the extent not superseded by documents or reports subsequently filed or furnished.

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### **Information Contained in this Form 6-K Report**

#### **Financial Statements**

This report contains the Company’s Unaudited Condensed Consolidated Interim Financial Statements as of and for the period ended June 30, 2024, including Management’s Discussion and Analysis of Financial Condition and Results of Operations for the period presented therein.

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## SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

### Ascendis Pharma A/S

Date: September 3, 2024

By: /s/ Michael Wolff Jensen

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Michael Wolff Jensen

Executive Vice President, Chief Legal Officer

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ASCENDIS PHARMA A/S

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**Unaudited Condensed Consolidated Interim Statements of Profit or (Loss)  
and Other Comprehensive Income or (Loss) for the Three and Six Months Ended June 30, 2024 and 2023**

	Notes	Three Months Ended June 30,		Six Months Ended June 30,	
		2024	2023	2024	2023
		(EUR'000)		(EUR'000)	
<b>Consolidated Statement of Profit or (Loss)</b>					
Revenue	5	35,998	47,393	131,892	80,982
Cost of sales		11,465	12,929	19,034	17,551
<b>Gross profit</b>		<b>24,533</b>	<b>34,464</b>	<b>112,858</b>	<b>63,431</b>
Research and development costs		83,478	105,021	154,165	211,134
Selling, general and administrative expenses		74,312	70,281	141,095	136,820
<b>Operating profit/(loss)</b>		<b>(133,257)</b>	<b>(140,838)</b>	<b>(182,402)</b>	<b>(284,523)</b>
Share of profit/(loss) of associates		(5,322)	(7,451)	(11,118)	(8,677)
Finance income		49,052	35,761	14,395	80,374
Finance expenses		19,624	9,334	58,553	18,652
<b>Profit/(loss) before tax</b>		<b>(109,151)</b>	<b>(121,862)</b>	<b>(237,678)</b>	<b>(231,478)</b>
Income taxes/(expenses)		(229)	429	(2,737)	(868)
<b>Net profit/(loss) for the period</b>		<b>(109,380)</b>	<b>(121,433)</b>	<b>(240,415)</b>	<b>(232,346)</b>
Attributable to owners of the Company		(109,380)	(121,433)	(240,415)	(232,346)
Basic and diluted earnings/(loss) per share		€ (1.91)	€ (2.16)	€ (4.21)	€ (4.14)
Number of shares used for calculation (basic and diluted) <sup>(1)</sup>		57,345,613	56,218,257	57,114,435	56,155,441
		(EUR'000)		(EUR'000)	
<b>Consolidated Statement of Comprehensive Income or (Loss)</b>					
<b>Net profit/(loss) for the period</b>		<b>(109,380)</b>	<b>(121,433)</b>	<b>(240,415)</b>	<b>(232,346)</b>
<b>Other comprehensive income/(loss)</b>					
<i>Items that may be reclassified subsequently to profit or (loss):</i>					
Exchange differences on translating foreign operations		15	(1,016)	78	(1,803)
<b>Other comprehensive income/(loss) for the period, net of tax</b>		<b>15</b>	<b>(1,016)</b>	<b>78</b>	<b>(1,803)</b>
<b>Total comprehensive income/(loss) for the period, net of tax</b>		<b>(109,365)</b>	<b>(122,449)</b>	<b>(240,337)</b>	<b>(234,149)</b>
Attributable to owners of the Company		(109,365)	(122,449)	(240,337)	(234,149)

<sup>(1)</sup> As of June 30, 2024 and June 30, 2023, a total of 6,107,875 and 6,866,241 warrants outstanding, respectively, each carrying the right to subscribe for one ordinary share, and 575,000 convertible senior notes which can potentially be converted into 3,456,785 ordinary shares, can potentially dilute earnings per share in the future but have not been included in the calculation of diluted earnings per share because they are antidilutive for the periods presented.

**Unaudited Condensed Consolidated Interim Statements of Financial Position**

	Notes	June 30, 2024	December 31, 2023
(EUR'000)			
<b>Assets</b>			
<b>Non-current assets</b>			
Intangible assets		4,186	4,419
Property, plant and equipment		104,041	110,634
Investment in associates	4	20,564	5,686
Other receivables	10	2,186	2,127
		<b>130,977</b>	<b>122,866</b>
<b>Current assets</b>			
Inventories		251,199	208,931
Trade receivables	10	49,163	35,874
Income tax receivables		1,841	802
Other receivables	10	29,679	19,097
Prepayments		36,743	38,578
Marketable securities	10	—	7,275
Cash and cash equivalents	10	258,696	392,164
		<b>627,321</b>	<b>702,721</b>
<b>Total assets</b>		<b>758,298</b>	<b>825,587</b>
<b>Equity and liabilities</b>			
<b>Equity</b>			
Share capital	8	7,819	7,749
Distributable equity		(328,952)	(153,446)
<b>Total equity</b>	4	<b>(321,133)</b>	<b>(145,697)</b>
<b>Non-current liabilities</b>			
Borrowings	2, 10	219,052	222,996
Contract liabilities		5,000	5,949
Deferred tax liabilities		7,644	5,830
		<b>231,696</b>	<b>234,775</b>
<b>Current liabilities</b>			
<i>Convertible notes, matures in April 2028</i>			
Borrowings	2, 10	432,190	407,095
Derivative liabilities	2, 10	159,059	143,296
		<b>591,249</b>	<b>550,391</b>
<i>Other current liabilities</i>			
Borrowings	2, 10	21,397	14,174
Contract liabilities		1,293	1,184
Trade payables and accrued expenses	10	99,527	94,566
Other liabilities	10	26,411	41,176
Income tax payables		1,090	2,299
Provisions	3	106,768	32,719
		<b>256,486</b>	<b>186,118</b>
		<b>847,735</b>	<b>736,509</b>
<b>Total liabilities</b>		<b>1,079,431</b>	<b>971,284</b>
<b>Total equity and liabilities</b>		<b>758,298</b>	<b>825,587</b>

**Unaudited Condensed Consolidated Interim Statements of Changes in Equity**

	Distributable Equity					Total
	Share Capital	Share Premium	Treasury Shares	Foreign Currency Translation Reserve	Accumulated Deficit	
	(EUR'000)					
<b>Equity at January 1, 2024</b>	7,749	2,123,074	(146)	721	(2,277,095)	(145,697)
Net profit/(loss) for the period	—	—	—	—	(240,415)	(240,415)
Other comprehensive income/(loss), net of tax	—	—	—	78	—	78
<b>Total comprehensive income/(loss)</b>	—	—	—	78	(240,415)	(240,337)
<b>Transactions with Owners</b>						
Share-based payment (Note 7)	—	—	—	—	43,327	43,327
Transfer under stock incentive programs	—	—	28	—	(28)	—
Capital increase	70	21,504	—	—	—	21,574
<b>Equity at June 30, 2024</b>	<b>7,819</b>	<b>2,144,578</b>	<b>(118)</b>	<b>799</b>	<b>(2,474,211)</b>	<b>(321,133)</b>
	Distributable Equity					
	Share Capital	Share Premium	Treasury Shares	Foreign Currency Translation Reserve	Accumulated Deficit	Total
	(EUR'000)					
<b>Equity at January 1, 2023</b>	7,675	2,112,863	(149)	3,452	(1,860,493)	263,348
Net profit/(loss) for the period	—	—	—	—	(232,346)	(232,346)
Other comprehensive income/(loss), net of tax	—	—	—	(1,803)	—	(1,803)
<b>Total comprehensive income/(loss)</b>	—	—	—	(1,803)	(232,346)	(234,149)
<b>Transactions with Owners</b>						
Share-based payment (Note 7)	—	—	—	—	33,568	33,568
Capital increase	24	2,050	—	—	—	2,074
<b>Equity at June 30, 2023</b>	<b>7,699</b>	<b>2,114,913</b>	<b>(149)</b>	<b>1,649</b>	<b>(2,059,271)</b>	<b>64,841</b>



**Unaudited Condensed Consolidated Interim Cash Flow Statements for the  
Six Months Ended June 30, 2024 and 2023**

	Six Months Ended June 30,	
	2024	2023
	(EUR'000)	
<b>Operating activities</b>		
<b>Net profit/(loss) for the period</b>	<b>(240,415)</b>	<b>(232,346)</b>
Reversal of finance income	(14,395)	(80,374)
Reversal of finance expenses	58,553	18,652
Reversal of (gain)/loss on disposal of property, plant and equipment	(91)	21
Reversal of income taxes (expenses)	2,737	868
Adjustments for non-cash items:		
Non-cash consideration relating to revenue	(25,639)	(1,203)
Share of profit/(loss) of associates	11,118	8,677
Share-based payment	43,327	33,568
Depreciation	8,632	8,969
Amortization	233	222
Changes in working capital:		
Inventories	(42,268)	(37,246)
Receivables	(22,747)	(10,715)
Prepayments	1,867	(12,779)
Contract liabilities (deferred income)	(840)	(9,118)
Trade payables, accrued expenses and other payables	(10,571)	9,316
Increase/(decrease) in provisions	72,276	3,625
<b>Cash flows generated from/(used in) operations</b>	<b>(158,223)</b>	<b>(299,863)</b>
Finance income received	6,265	8,403
Finance expenses paid	(7,714)	(7,746)
Income taxes received/(paid)	(3,218)	(966)
<b>Cash flows from/(used in) operating activities</b>	<b>(162,890)</b>	<b>(300,172)</b>
<b>Investing activities</b>		
Acquisition of property, plant and equipment	(541)	(1,529)
Proceeds from disposal of property, plant and equipment	950	—
Settlement of marketable securities	7,354	259,132
<b>Cash flows from/(used in) investing activities</b>	<b>7,763</b>	<b>257,603</b>
<b>Financing activities</b>		
Repayment of borrowings	(5,606)	(5,085)
Proceeds from exercise of warrants	21,574	2,074
<b>Cash flows from/(used in) financing activities</b>	<b>15,968</b>	<b>(3,011)</b>
<b>Increase/(decrease) in cash and cash equivalents</b>	<b>(139,159)</b>	<b>(45,580)</b>
Cash and cash equivalents at January 1	392,164	444,767
Effect of exchange rate changes on balances held in foreign currencies	5,691	(4,965)
<b>Cash and cash equivalents at June 30</b>	<b>258,696</b>	<b>394,222</b>
<b>Cash and cash equivalents include:</b>		
Bank deposits	258,696	394,222
<b>Cash and cash equivalents at June 30</b>	<b>258,696</b>	<b>394,222</b>

## Notes to the Unaudited Condensed Consolidated Interim Financial Statements

### Note 1—General Information

Ascendis Pharma A/S, together with its subsidiaries, is applying its innovative TransCon technology platform to build a leading, fully integrated, global biopharma company focused on making a meaningful difference in patients' lives. Ascendis Pharma A/S was incorporated in 2006 and is headquartered in Hellerup, Denmark. Unless the context otherwise requires, references to the "Company," "we," "us," and "our," refer to Ascendis Pharma A/S and its subsidiaries.

The address of the Company's registered office is Tuborg Boulevard 12, DK-2900, Hellerup, Denmark.

On February 2, 2015, the Company completed an initial public offering which resulted in the listing of American Depositary Shares ("ADSs"), representing the Company's ordinary shares, under the symbol "ASND" in the United States on The Nasdaq Global Select Market.

The Company's Board of Directors (the "Board") approved these unaudited condensed consolidated interim financial statements on September 3, 2024.

### Note 2—Summary of Material Accounting Policies

#### *Basis of Preparation*

The unaudited condensed consolidated interim financial statements of the Company are prepared in accordance with International Accounting Standard 34, "Interim Financial Reporting." Certain information and disclosures normally included in the annual consolidated financial statements prepared in accordance with IFRS Accounting Standards ("IFRS") have been condensed or omitted. Accordingly, these unaudited condensed consolidated interim financial statements should be read in conjunction with the Company's audited annual consolidated financial statements for the year ended December 31, 2023, and accompanying notes, which have been prepared in accordance with IFRS as issued by the International Accounting Standards Board (the "IASB") and as adopted by the European Union (the "EU").

The accounting policies applied are consistent with those of the previous financial year. A description of the accounting policies is provided in the Accounting Policies section of the audited consolidated financial statements as of and for the year ended December 31, 2023.

#### *New and Amended Standards and Interpretations*

The Company has applied amendments to paragraphs 69 to 76 of IAS 1, "Presentation of Financial Statements," which was effective for annual reporting periods beginning on or after January 1, 2024, and must be applied retrospectively. The amendments to IAS 1 specify the requirements for classifying liabilities as current or non-current. The amendments clarify:

- What is meant by a right to defer settlement;
- That a right to defer must exist at the end of the reporting period;
- That classification is unaffected by the likelihood that an entity will exercise its deferral right; and
- That only if an embedded derivative in a convertible liability is itself an equity instrument would the terms of a liability not impact its classification.

The convertible senior notes ("convertible notes") include an embedded equity conversion option which is not deemed closely related to the financial liability and was initially recognized and measured separately at fair value as derivative liabilities based on the stated terms upon issuance of the convertible notes. The conversion option is classified as a foreign currency conversion option and thus not convertible into a fixed number of shares for a fixed amount of cash. Accordingly, the conversion option is subsequently recognized and measured as a derivative liability at fair value through profit or loss, with any subsequent remeasurement gains or losses recognized as part of finance income or expenses.

Since the embedded derivative is not an equity instrument under IFRS, the amendments require the convertible notes (presented as part of borrowings in the statement of financial position) and derivative liabilities, presented as non-current liabilities at December 31, 2023, to be presented as current liabilities. The amendments require presentation of the convertible notes as current liabilities even though: the initial conversion price of \$166.34 per ADS is not met; the conversion would not require cash settlement; and, the convertible notes do not mature until April 1, 2028. Further details, including (cash) maturity analysis are provided in Note 10, “Financial Assets and Liabilities.” On December 31, 2023, the carrying amount of convertible notes and derivative liabilities were €407.1 million and €143.3 million, respectively. Comparative amounts have been reclassified to reflect the change to presentation.

On December 31, 2023, lease liabilities were presented separately in the consolidated statements of financial position. At December 31, 2023, carrying amount of lease liabilities was €84.6 million and €14.2 million, for non-current liabilities and current liabilities, respectively. Since March 31, 2024, lease liabilities are presented as part of borrowings in the consolidated statements of financial position. Comparative amounts have been reclassified to reflect the change in presentation.

Accordingly, since March 31, 2024, borrowings comprise convertible notes, royalty funding liabilities, and lease liabilities. The change to presentation had no other impact on the unaudited condensed consolidated financial statements.

The applied amendments had no other impact on the unaudited condensed consolidated interim financial statements.

Other amendments apply for the first time in 2024, but do not have an impact on the unaudited condensed consolidated interim financial statements.

### ***New International Financial Reporting Standards Not Yet Effective***

The IASB has issued a number of new or amended standards, which have not yet become effective or have not yet been adopted by the EU. Therefore, these new standards have not been incorporated in these unaudited condensed consolidated interim financial statements.

### ***IFRS 18, “Presentation and Disclosure in Financial Statements”***

In April 2024, the IASB issued IFRS 18, “Presentation and Disclosure in Financial Statements” (“IFRS 18”), which replaces IAS 1, “Presentation in Financial Statements.” IFRS 18 introduces new categories and subtotals in the statement of profit or loss, into:

- Operating activities;
- Investing activities;
- Financing activities;
- Income taxes; and
- Discontinued operations.

In addition, IFRS 18 includes new requirements for the location, aggregation and disaggregation of financial information, and disclosure of management-defined performance measures, as defined, if any. IFRS 18 does not include any measurement changes.

If approved by the EU, the amendments will be effective for annual reporting periods beginning on or after January 1, 2027, and must be applied retrospectively, with early adoption permitted. While IFRS 18 will change the structure and subtotal in the statement of profit or loss, the full impact from implementing IFRS 18 is currently being analyzed by the Company.

The consolidated financial statements are not expected to be affected by other new or amended standards.

### **Note 3—Significant Accounting Judgements and Estimates**

In the application of the Company's accounting policies, management is required to make judgements, estimates and assumptions about the carrying amounts of assets and liabilities that are not readily apparent from other sources. Judgements, estimates and assumptions applied are based on historical experience and other factors that are relevant, and which are available at the reporting date. Uncertainty concerning estimates and assumptions could result in outcomes that require a material adjustment to assets and liabilities in future periods.

The unaudited condensed consolidated interim financial statements do not include all disclosures for significant accounting judgements, estimates and assumptions, that are required in the annual consolidated financial statements, and therefore should be read in conjunction with the Company's audited consolidated financial statements as of and for the year ended December 31, 2023.

Estimates and underlying assumptions are reviewed on an ongoing basis. Revisions to accounting estimates are recognized prospectively. While the application of critical accounting estimates is subject to material estimation uncertainties, management's ongoing revisions of critical accounting estimates and underlying assumptions have, except for revenue and provisions, as described below, not revealed any material impact in any of the periods presented in the unaudited condensed consolidated interim financial statements.

There have been no other changes to the application of significant accounting judgements, or estimation uncertainties regarding accounting estimates compared to December 31, 2023.

#### ***Revenue and Provisions - Change in Accounting Estimates***

Revenue includes provision for a variety of sales deductions such as prompt pay discounts, shelf stock adjustments and applicable sales rebates attributable to various commercial arrangements, managed healthcare organizations, government programs, and co-pay arrangement. Provisions for sales deductions attributed to commercial arrangements are recognized when the related sales takes place and measured using the expected value method. Payable amounts for commercial arrangements are generally settled within 90-180 days from the transaction date. Provisions for unsettled sales deductions under commercial arrangements are estimated on the basis of a percentage of sales as defined by individual agreements and contracts. Further inputs to the calculations are based on payer channel mix, current contract prices under eligible programs and current inventory levels in the distribution channels. Inputs to the calculations are subject to estimation and assumptions and are based on historical experience and other factors that are relevant, and which are available at the reporting date. These estimates and assumptions are subject to material uncertainties and could result in outcomes that require a material adjustment in future periods.

Revenue for the three months ended June 30, 2024, was negatively impacted by an adjustment to prior periods estimates and assumptions for sales deductions of €27.1 million, where €19.5 million and €7.6 million were attributable to the three months ended March 31, 2024 and periods prior to January 1, 2024, respectively. The adjustment was primarily attributable to a different payer and rebate mix than anticipated, and which provisions for prior periods were based upon.

Total provisions for the year ended, December 31, 2023 and six months ended June 30, 2024, were €32.7 million and €106.8 million, respectively.

### **Note 4—Significant Events in the Reporting Period**

#### ***Eyconis, Inc.***

On January 29, 2024, the Company announced the formation and launch with Frazier Life Sciences of Eyconis, Inc. ("Eyconis"), a separate company created to develop, manufacture, and commercialize TransCon ophthalmology assets globally, together with a \$150 million commitment from an investor syndicate that includes Frazier, RA Capital Management, venBio, and HealthQuest Capital.

The Company has granted Eyconis exclusive rights to develop and commercialize TransCon ophthalmology products globally and received an equity position in the newly formed company. As consideration for the granting of such rights, the Company received, as consideration, approximately 42% ownership of Eyconis' on a non-diluted basis. In addition, various development and administrative services were provided to Eyconis and invoiced during the three and six months ended June 30, 2024. Further details regarding Eyconis are provided in Note 5, "Revenue."

### ***Equity Development***

As of June 30, 2024, the unaudited condensed consolidated interim statements of financial position presented a negative balance of equity of €321.1 million. Under Danish corporate law, as Ascendis Pharma A/S, the parent company of the Company holds a positive balance of equity, the Company is currently not subject to legal or regulatory requirements to re-establish the balance of equity. There is no direct impact from the negative balance of equity to the liquidity and capital resources.

Based on its current operating plan, the Company believes that the existing capital resources as of June 30, 2024, will be sufficient to meet projected cash requirements for at least twelve months from the date of this report. However, the Company's operating plan may change as a result of many factors that are currently unknown, and the Company may need to seek additional funds sooner than planned. Further details regarding borrowings including maturity analysis are provided in Note 10, "Financial Assets and Liabilities."

## Note 5—Revenue

Revenue has been recognized in the unaudited condensed consolidated interim statements of profit or loss in the following amounts:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
	(EUR'000)		(EUR'000)	
<b>Revenue</b>				
Sale of commercial products	31,389	35,895	97,888	67,446
Rendering of services	3,740	10,909	8,364	12,079
Sale of clinical supply	—	—	1	254
Licenses	869	589	25,639	1,203
<b>Total revenue</b>	<b>35,998</b>	<b>47,393</b>	<b>131,892</b>	<b>80,982</b>
<b>Attributable to</b>				
Commercial customers	31,389	35,895	97,888	67,446
Collaboration partners and license agreements	4,609	11,498	34,004	13,536
<b>Total revenue</b>	<b>35,998</b>	<b>47,393</b>	<b>131,892</b>	<b>80,982</b>
<b>Specified by timing of recognition</b>				
Recognized over time	3,740	10,909	8,364	12,079
Recognized at a point in time	32,258	36,484	123,528	68,903
<b>Total revenue</b>	<b>35,998</b>	<b>47,393</b>	<b>131,892</b>	<b>80,982</b>
<b>Specified per geographical area</b>				
Europe	5,082	—	6,649	—
North America	29,394	46,758	122,075	79,828
Asia	1,522	635	3,168	1,154
<b>Total revenue</b>	<b>35,998</b>	<b>47,393</b>	<b>131,892</b>	<b>80,982</b>

### Commercial Customers

Revenue to commercial customers primarily relates to sale of SKYTROFA<sup>®</sup> (lonapegsomatropin-tcgd), primarily in the U.S. market, which is sold to specialty pharmacies and specialty distributors. In addition, the Company began shipping SKYTROFA to customers in Germany in September 2023. In November 2023, TransCon PTH received regulatory approval in the EU and European Economic Area countries and is marketed as YORVIPATH<sup>®</sup> (palopegteriparatide). In August 2024, TransCon PTH received regulatory approval in the U.S. and will be marketed in the U.S. as YORVIPATH. The Company began shipping YORVIPATH to customers in Europe in the first quarter of 2024.

For the three and six months ended June 30, 2024 and 2023, four commercial customers, each represented more than 10% of sales to commercial customers.

### Collaboration Partners and License Agreements

Revenue attributable to collaboration partners and license agreements relates to Eyconis, Teijin Limited and VISEN Pharmaceuticals. Under the collaboration agreements, we provide various research and development services which are invoiced to collaboration partners. Revenue for these activities is presented as part of “Rendering of services.” Employment costs related to these activities are presented as Research and Development Costs in the consolidated statement of profit or loss.

#### *Eyconis, Inc.*

On January 29, 2024, the Company announced the formation and launch with Frazier Life Sciences of Eyconis, Inc., a separate company created to develop, manufacture, and commercialize TransCon ophthalmology assets globally, together with a \$150 million commitment from an investor syndicate that includes Frazier, RA Capital Management, venBio, and HealthQuest Capital.

The Company has granted Eyconis exclusive rights to develop and commercialize TransCon ophthalmology products globally and received, as consideration, an equity position in the newly formed company. In addition, the Company is eligible to receive development, regulatory, and sales milestone payments, plus single digit royalties on global net sales of commercialized products, if any.

The Company is expected to provide various research and development services, which are subject to separate remuneration, and which will be recognized as revenue over time as rendering of services or reimbursement revenue, as applicable.

For the six months ended June 30, 2024, revenue from “Licenses” of €25.6 million relates to non-cash upfront payment through an equity position in Eyconis, which is allocated to transfer of the Company’s intellectual property (“IP”) adjusted for internal profit. Internal profit relates to the Company’s share of the non-cash upfront payment which is recognized as part of “Investment in associates” and recognized as revenue from “Licenses” as the IP is amortized in the associate.

For the six months ended June 30, 2024, no revenue from royalties or milestones has been recognized under the Eyconis agreement.

#### *Teijin Limited*

On November 29, 2023, the Company entered into an exclusive license agreement with Teijin Limited (the “Teijin Agreement”) for the further development and commercialization of TransCon hGH, TransCon PTH, and TransCon CNP for endocrinology rare disease (the “Licensed Products”) in Japan. Under the terms of the Teijin Agreement, the Company received an upfront payment of \$70 million, with additional development and regulatory milestones of up to \$175 million and commercial milestones. In addition, the Company is eligible to receive royalties on net sales of the Licensed Products in Japan, of up to mid-20’s percent.

Further, the Company will provide clinical and commercial supply, and development services for joint activities, which are subject to separate remuneration, and which will be recognized as revenue over time as rendering of services or reimbursement revenue, as applicable.

At June 30, 2024, none of the Licensed Products have received marketing authorization in Japan. The Licensed Products are patent protected, where future activities do not affect their existing stand-alone functionalities. Accordingly, all three licenses have been classified as “right-to-use” licenses, with revenue recognized at a point in time, where the licensee is granted access to the IP.

Development and regulatory milestones of up to \$175 million are recognized as revenue when the milestone criteria specific to the Licensed Product are met. Royalty and commercial milestone income is recognized as revenue when the subsequent product sales occur.

For the six months ended June 30, 2024, no revenue from royalties or milestones has been recognized under the Teijin Agreement.

#### *VISEN Pharmaceuticals*

Revenue from collaboration partners and license agreements also includes license income, rendering of services and sale of clinical supply under three licenses agreements with VISEN Pharmaceuticals, which were entered into in 2018.

#### **Note 6—Segment Information**

The Company is managed and operated as one business unit. Accordingly, no additional information on business segments or geographical areas is disclosed apart from revenue on geographical areas as disclosed in Note 5 “Revenue.” Revenue is specified on geographical areas according to the location of the customer.

#### **Note 7—Share-based Payment**

As an incentive to the senior management, other employees, members of the Board and select consultants, Ascendis Pharma A/S has established warrant programs, a Restricted Stock Unit (“RSU”) program adopted in December 2021, and a Performance Stock Unit (“PSU”) program adopted in February 2023, which are all classified as equity-settled share-based payment transactions.

#### **Share-based Compensation Costs**

Share-based compensation costs are determined using the grant date fair value and are recognized over the vesting period as research and development costs, selling, general and administrative expenses, or cost of sales. For the three and six months ended June 30, 2024 and 2023, share-based compensation costs recognized in the unaudited condensed consolidated interim statement of profit or loss were €26.0 million and €43.3 million, respectively and €19.9 million and €33.6 million, respectively.

#### **Restricted Stock Unit Program**

RSUs are granted by the Board to members of senior management, certain other employees and certain members of the Board (the “RSU-holders”). In addition, RSUs may be granted to select consultants.

One RSU represents a right for the RSU-holder to receive one ADS representing ordinary shares of Ascendis Pharma A/S upon vesting, if the vesting conditions are met. RSUs granted vest over three years with 1/3 of the RSUs vesting on each anniversary date from the date of grant, and require RSU-holders to be employed, appointed as member of the Board, or retained as a consultant (the “service conditions”).

### ***Performance Stock Unit Program***

PSUs are granted by the Board to certain members of senior management (the “PSU-holders”). In addition, PSUs may be granted to other employees, select consultants and members of the Board.

One PSU represents a right for the PSU-holder to receive one ADS representing ordinary shares of Ascendis Pharma A/S upon vesting. PSUs vest in a manner similar to the service conditions of the RSUs. For the March 2023 PSU grant, in addition to service conditions, vesting is also contingent upon achievement of performance-based targets as determined by the Board, provided that no more than 10% of each tranche may be directly attributable to accomplishment of financial results achieved in the financial year prior to the vesting date. For the March 2024 PSU grants, in addition to service conditions, vesting is also contingent upon achievement of long-term strategic goals as evaluated by the Board no later than two weeks prior to each vesting date. Exceeding performance targets will not result in vesting of more PSUs than 100%, nor will it result in additional grants.

RSUs and PSUs generally cease to vest from the date of termination of employment or Board membership, as applicable, whereas unvested RSUs or PSUs will forfeit. The Board may at its discretion and on an individual basis decide to deviate from the vesting conditions, including deciding to accelerate vesting in the event of termination of employment or Board membership, as applicable.

All RSUs and PSUs are expected to be settled at the time of vesting by treasury shares that are ADSs repurchased by the Company. The Company may at its sole discretion choose to make a cash settlement instead of delivering ADSs.

### ***RSU and PSU Activity***

The following table specifies the number of RSUs and PSUs granted and outstanding at June 30, 2024:

	Restricted Stock Units	Performance Stock Units	Total
<b>Outstanding</b>		(Number)	
January 1, 2024	576,625	105,023	681,648
Granted during the period	717,980	92,655	810,635
Transferred during the period	(176,317)	(35,007)	(211,324)
Forfeited during the period	(63,309)	(4,830)	(68,139)
<b>June 30, 2024</b>	<b>1,054,979</b>	<b>157,841</b>	<b>1,212,820</b>
<b>Specified by vesting year</b>			
2024	35,843	—	35,843
2025	393,254	63,478	456,732
2026	393,612	63,478	457,090
2027	232,270	30,885	263,155
<b>June 30, 2024</b>	<b>1,054,979</b>	<b>157,841</b>	<b>1,212,820</b>

### ***Warrant Program***

Warrants are granted by the Board in accordance with authorizations given to it by the shareholders of Ascendis Pharma A/S to all employees, members of the Board and select consultants. Each warrant carries the right to subscribe for one ordinary share of a nominal value of DKK 1. The exercise price is fixed at the fair market value of the Company’s ordinary shares at the time of grant as determined by the Board. Vested warrants may be exercised in two or four annual exercise periods.



### Warrant Activity

The following table specifies the warrant activity for the six months ended June 30, 2024:

	Total Warrants	Weighted Average Exercise Price
	(Number)	(EUR)
<b>Outstanding</b>		
January 1, 2024	6,523,784	86.38
Granted during the period	197,855	130.74
Exercised during the period	(524,045)	39.85
Forfeited during the period	(89,719)	108.71
<b>June 30, 2024</b>	<b>6,107,875</b>	<b>91.16</b>
<b>Vested at June 30, 2024</b>	<b>5,088,475</b>	<b>86.49</b>

The exercise prices of outstanding warrants under the Company's warrant programs range from €11.98 to €145.50 depending on the grant dates.

### Note 8—Share Capital

The share capital of Ascendis Pharma A/S consists of 58,231,484 fully paid shares at a nominal value of DKK 1, all in the same share class.

### Note 9—Treasury Shares

The development in the holding of treasury shares is as follows:

	Nominal values	Holding	Holding in % of total outstanding shares
	(EUR'000)	(Number)	
<b>Treasury shares</b>			
January 1, 2024	146	1,093,054	1.9%
Transferred under stock incentive programs	(28)	(211,324)	—
<b>June 30, 2024</b>	<b>118</b>	<b>881,730</b>	<b>1.5%</b>

## Note 10—Financial Assets and Liabilities

The following table specifies financial assets and liabilities:

	June 30, 2024	December 31, 2023
	(EUR'000)	
<b>Financial assets by category</b>		
Trade receivables	49,163	35,874
Other receivables (excluding income tax and indirect tax receivables)	5,062	3,909
Marketable securities	—	7,275
Cash and cash equivalents	258,696	392,164
<b>Financial assets measured at amortized cost</b>	<b>312,921</b>	<b>439,222</b>
<b>Total financial assets</b>	<b>312,921</b>	<b>439,222</b>
<b>Classified in the statement of financial position</b>		
Non-current assets	2,186	2,127
Current assets	310,735	437,095
<b>Total financial assets</b>	<b>312,921</b>	<b>439,222</b>
<b>Financial liabilities by category</b>		
Borrowings		
Convertible senior notes	432,190	407,095
Royalty funding liabilities	144,547	138,377
Lease liabilities	95,902	98,793
Trade payables and accrued expenses	99,527	94,566
Other liabilities (excluding income tax, indirect tax, and employee related payables)	302	—
<b>Financial liabilities measured at amortized cost</b>	<b>772,468</b>	<b>738,831</b>
Derivative liabilities	159,059	143,296
<b>Financial liabilities measured at fair value through profit or loss</b>	<b>159,059</b>	<b>143,296</b>
<b>Total financial liabilities</b>	<b>931,527</b>	<b>882,127</b>
<b>Classified in the statement of financial position</b>		
Non-current liabilities	219,052	222,996
Current liabilities	712,475	659,131
<b>Total financial liabilities</b>	<b>931,527</b>	<b>882,127</b>

### Convertible Senior Notes

In March 2022, the Company issued an aggregate principal amount of \$575.0 million of fixed rate 2.25% convertible notes. The net proceeds from the offering of the convertible notes were \$557.9 million (€503.3 million) after deducting the initial purchasers' discounts and commissions and offering expenses. The convertible notes rank equally in right of payment with all future senior unsecured indebtedness. Unless earlier converted or redeemed, the convertible notes will mature on April 1, 2028.

The convertible notes accrue interest at a rate of 2.25% per annum, payable semi-annually in arrears on April 1 and October 1 of each year. At any time before the close of business on the second scheduled trading day immediately before the maturity date, noteholders may convert their convertible notes at their option into the Company's ordinary shares represented by ADSs, together, if applicable, with cash in lieu of any fractional ADS, at the then-applicable conversion rate. The initial conversion rate is 6.0118 ADSs per \$1,000 principal amount of convertible notes, which represents an initial conversion price of \$166.34 per ADS. The conversion rate and conversion price will be subject to customary adjustments upon the occurrence of certain events.

The convertible notes will be optionally redeemable, in whole or in part (subject to certain limitations), at the Company's option at any time, and from time to time, on or after April 7, 2025, but only if the last reported sale price per ADS exceeds 130% of the conversion price on (i) each of at least 20 trading days, whether or not consecutive, during the 30 consecutive trading days ending on, and including, the trading day immediately before the date the Company sends the related optional redemption notice; and (ii) the trading day immediately before the date the Company sends such notice.

On June 30, 2024, the carrying amount of the convertible notes was €432.2 million, and the fair value was approximately €412.1 million. Fair value cannot be measured based on quoted prices in active markets or other observable input, and accordingly the fair value was measured by using an estimated market rate for an equivalent non-convertible instrument.

### ***Royalty Funding Liabilities***

In September 2023, the Company entered into a \$150.0 million capped synthetic royalty funding agreement (the “Royalty Pharma Agreement”) with Royalty Pharma (the “Purchaser”). The net proceeds were \$146.3 million (€136.3 million) after deducting offering expenses.

Under the terms of the Royalty Pharma Agreement, the Company received an upfront payment of \$150.0 million (the “Purchase Price”) in exchange for a 9.15% royalty on net U.S. SKYTROFA revenue, beginning on January 1, 2025 (the “Revenue Interest Payments”). The Revenue Interest Payments to the Purchaser will cease upon reaching a multiple of the Purchase Price of 1.925x, or 1.65x if the Purchaser receives Revenue Interest Payments in that amount by December 31, 2031.

The Royalty Pharma Agreement includes a buy-out option, which provides the Company with the right to settle all outstanding liabilities at any time by paying a buy-out amount equal to 1.925 times the Purchase Price minus the Revenue Interest Payments paid to the Purchaser as of the effective date of the buy-out notice. However, if the buy-out notice is provided on or prior to December 31, 2028, and the Company has paid the Purchaser Revenue Interest Payments equal to the Purchase Price as of the date of the buy-out notice, then the buy-out amount equal to 1.65 times the Purchase Price minus the Revenue Interest Payments paid to the Purchaser as of the effective date of the buy-out notice.

On June 30, 2024, the carrying amount of the royalty funding liabilities was €144.5 million, and the fair value was approximately €147.3 million. Fair value cannot be measured based on quoted prices in active markets or other observable input, and accordingly the fair value was measured by using an estimated market rate for an equivalent instrument.

### ***Derivative Liabilities***

Derivative liabilities relate to the foreign currency conversion option embedded in the convertible notes.

Fair value cannot be measured based on quoted prices in active markets or other observable inputs, and accordingly, derivative liabilities are measured by using the Black-Scholes option pricing model. Fair value of the option is calculated, applying the following assumptions: (1) conversion price; (2) the Company’s share price; (3) maturity of the option; (4) a risk-free interest rate equaling the effective interest rate on a U.S. government bond with the same lifetime as the maturity of the option; (5) no payment of dividends; and (6) an expected volatility using the Company’s share price (49.89% and 50.47% as of June 30, 2024 and December 31, 2023, respectively).

For additional description of fair values, refer to the following section “Fair Value Measurement.”

### ***Sensitivity Analysis***

On June 30, 2024, all other inputs and assumptions held constant, a 10% relative increase in volatility, will increase the fair value of derivative liabilities by approximately €15.2 million and indicates a decrease in profit or loss and equity before tax. Similarly, a 10% relative decrease in volatility indicates the opposite impact.

Similarly, on June 30, 2024, all other inputs and assumptions held constant, a 10% increase in the share price, will increase the fair value of derivative liabilities by approximately €30.5 million and indicates a decrease in profit or loss and equity before tax. Similarly, a 10% decrease in the share price indicates the opposite impact.

### ***Fair Value Measurement***

Because of the short-term maturity for cash and cash equivalents, receivables and trade payables, their fair value approximate carrying amount. Fair value of lease liabilities are not disclosed. Fair value compared to carrying amount of marketable securities, convertible notes, royalty funding liabilities and derivative liabilities, and their level in the fair value hierarchy is summarized in the following table, where:

**Level 1** is quoted prices (unadjusted) in active markets for identical assets or liabilities that the entity can access at the measurement date;

**Level 2** is based on valuation techniques for which the lowest level input that is significant to the fair value measurement is directly or indirectly observable;

**Level 3** is based on valuation techniques for which the lowest level input that is significant to the fair value measurement is unobservable.

	June 30, 2024		December 31, 2023		Fair value level (1-3)
	Carrying amount	Fair value	Carrying amount	Fair value	
	(EUR'000)				
<b>Financial assets</b>					
Marketable securities	—	—	7,275	7,266	1
<b>Financial assets measured at amortized cost</b>	<b>—</b>	<b>—</b>	<b>7,275</b>	<b>7,266</b>	
<b>Financial liabilities</b>					
Convertible senior notes	432,190	412,122	407,095	385,410	3
Royalty funding liabilities	144,547	147,345	138,377	143,975	3
<b>Financial liabilities measured at amortized cost</b>	<b>576,737</b>	<b>559,467</b>	<b>545,472</b>	<b>529,385</b>	
Derivative liabilities	159,059	159,059	143,296	143,296	3
<b>Financial liabilities measured at fair value through profit or loss</b>	<b>159,059</b>	<b>159,059</b>	<b>143,296</b>	<b>143,296</b>	

The following table specifies movements in level 3 fair value measurements:

	2024	2023
	(EUR'000)	
<b>Derivative liabilities</b>		
January 1	143,296	157,950
Remeasurement recognized in finance (income) or expense	15,763	(71,565)
<b>June 30</b>	<b>159,059</b>	<b>86,385</b>

### *Maturity Analysis*

The following table summarizes maturity analysis (on an undiscounted basis) for non-derivative financial liabilities recognized in the unaudited condensed consolidated statements of financial position at June 30, 2024. Further details regarding classification of convertible notes as current liabilities in the consolidated statement of financial position are provided in Note 2, “Summary of Material Accounting Policies,” section “New and Amended Standards and Interpretations.”

	< 1 year	1-5 years	>5 years	Total contractual cash-flows	Carrying amount
	(EUR'000)				
<b>Financial liabilities</b>					
<b>June 30, 2024</b>					
Borrowings (excluding lease liabilities)	18,902	759,073	38,700	816,675	576,737
Lease liabilities	14,802	51,632	44,312	110,746	95,902
Trade payables, accrued expenses and other liabilities	99,829	—	—	99,829	99,829
<b>Total financial liabilities</b>	<b>133,533</b>	<b>810,705</b>	<b>83,012</b>	<b>1,027,250</b>	<b>772,468</b>

**Note 11—Subsequent Events**

In September 2024, the Company entered into a \$150 million capped synthetic royalty funding agreement (the “Yorvipath Agreement”) with Royalty Pharma (the “Purchaser”). Under the terms of the Yorvipath Agreement, Purchaser has agreed to provide the Company an upfront payment of \$150 million (the “Purchase Amount”) in exchange for a 3% royalty on net U.S. YORVIPATH revenue (the “Royalty Payments”). The Royalty Payments to the Purchaser will cease upon reaching a multiple of the Purchase Amount of 2.0x, or 1.65x if the Purchaser receives royalties in that amount by December 31, 2029. The Yorvipath Agreement includes certain buy-out options under various terms and conditions.

No other events have occurred after the balance sheet date that would influence the evaluation of these unaudited condensed consolidated interim financial statements.

## ASCENDIS PHARMA A/S

### MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

*You should read the following discussion and analysis of our financial condition and results of operations in conjunction with our unaudited condensed consolidated interim financial statements, including the notes thereto, included with this report and the section contained in our Annual Report on Form 20-F for the year ended December 31, 2023– “Item 5. Operating and Financial Review and Prospects.” The following discussion is based on our financial information prepared in accordance with International Accounting Standard 34, “Interim Financial Reporting.” Certain information and disclosures normally included in the consolidated financial statements prepared in accordance with IFRS Accounting Standards (“IFRS”) have been condensed or omitted. IFRS as issued by the International Accounting Standards Board, and as adopted by the European Union, might differ in material respects from generally accepted accounting principles in other jurisdictions.*

#### **Special Note Regarding Forward-Looking Statements**

This report contains forward-looking statements concerning our business, operations and financial performance and condition, as well as our plans, objectives and expectations for our business operations and financial performance and condition. Any statements contained herein that are not statements of historical facts may be deemed to be forward-looking statements. In some cases, you can identify forward-looking statements by terminology such as “aim,” “anticipate,” “assume,” “believe,” “contemplate,” “continue,” “could,” “due,” “estimate,” “expect,” “goal,” “intend,” “may,” “objective,” “plan,” “predict,” “potential,” “positioned,” “seek,” “should,” “target,” “will,” “would,” and other similar expressions that are predictions or indicate future events and future trends, or the negative of these terms or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- the timing or likelihood of regulatory filings and approvals for our product candidates;
- our expectations regarding the commercial availability of our approved products, in the United States, European countries, and related patient support services;
- the commercialization of our products and product candidates, if approved;
- our commercialization, marketing and manufacturing capabilities of our products and product candidates and associated devices;
- the scope, timing, progress, results and costs of developing our product candidates or any other future product candidates, and conducting preclinical studies and clinical trials;
- our pursuit of oncology as our second independent therapeutic area of focus and our development of a pipeline of product candidates related to oncology;
- Eyconis, Inc.’s ability to develop, manufacture, and commercialize TransCon ophthalmology assets globally;
- our expectations regarding the potential market opportunities and patient populations for our products and product candidates, if approved for commercial use;
- our expectations regarding the potential advantages of our products and product candidates over existing therapies;
- the potential benefits of using our products and product candidates in combination with each other and other therapies;
- our ability to enter into new collaborations;
- our expectations with regard to the ability to develop additional product candidates using our TransCon technologies and submit Investigational New Drug Applications (“INDs”) or similar for such product candidates;
- our expectations with regard to our current and future collaboration partners to pursue the development of our product candidates and submit INDs or similar for such product candidates;
- our development plans with respect to our products and product candidates;
- our pursuit of additional indications for TransCon hGH;
- the implementation of our business model and strategic plans for our business, our products and product candidates and technologies, including global commercialization strategies;

- the scope of protection we are able to establish and maintain for intellectual property rights covering our products and product candidates;
- our expectations regarding our ability to apply our technology platform and algorithm for product innovation to develop highly differentiated product candidates to address unmet medical needs;
- our ability to apply our TransCon technology platform to build a leading, fully integrated, global biopharma company;
- our use of our TransCon technologies to create new and potentially best-in-class therapies;
- our ability to achieve our goals for Vision 2030;
- estimates of our expenses, future revenue, capital requirements, needs for additional financing and ability to obtain additional capital;
- our financial performance;
- our ability to attract and hire qualified personnel;
- developments and projections relating to market conditions, competitors and the industry;
- the impact of international economic, political, legal, compliance, social and business factors, including inflation, geopolitical conflicts and energy shortages; and
- the effects on our business of pandemics and the ongoing conflicts in the region surrounding Ukraine and Russia and between Israel and Hamas.

These forward-looking statements are based on senior management’s current expectations, estimates, forecasts and projections about our business and the industry in which we operate and involve known and unknown risks, uncertainties and other factors that are in some cases beyond our control. As a result, any or all of our forward-looking statements in this report may turn out to be inaccurate. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under the section in our Annual Report on Form 20-F for the year ended December 31, 2023 — “Item 3.D. Risk Factors.” You are urged to consider these factors carefully in evaluating the forward-looking statements. These forward-looking statements speak only as of the date of this report. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future. Given these risks and uncertainties, you are cautioned not to rely on such forward-looking statements as predictions of future events.

You should read this report and the documents that we reference in this report and have filed as exhibits to this report completely and with the understanding that our actual future results may be materially different from what we expect. You should also review the factors and risks we describe in the reports we will file or submit from time to time with the Securities and Exchange Commission after the date of this report. We qualify all of our forward-looking statements by these cautionary statements.

## Overview

We are applying our innovative TransCon technology platform to build a leading, fully integrated, global biopharma company focused on making a meaningful difference in patients’ lives. Guided by our core values of Patients, Science, and Passion, we use our TransCon technologies to create new and potentially best-in-class therapies.

## Our Vision

As announced in January 2024, Vision 2030 is our vision to achieve blockbuster status for multiple products and expand our engine for future innovation. This includes:

- Be the Leading Endocrinology Rare Disease Company
  - o Achieve blockbuster status (>\$1B) for each of TransCon PTH, TransCon hGH, and TransCon CNP through worldwide commercialization
  - o Be the leader in growth disorders and hypoparathyroidism, pursuing clinical conditions, innovative life cycle management, and complementary patient offerings
  - o Expand pipeline with Endocrinology Rare Disease blockbuster product opportunities
- Create Value in Additional Therapeutic Areas through Innovative Business Models
  - o Obtain accelerated approval in oncology with registrational trials ongoing

- o Pursue TransCon product opportunities in >\$5B indications
- o Maximize value creation of these product opportunities through collaboration with therapeutic area market leaders
- Differentiate with Ascendis Fundamentals
  - o Outperform industry drug development benchmarks with Ascendis’ product innovation algorithm
  - o Remain independent as a profitable biopharma through lean and flexible ways of working
  - o Let our values Patients, Science, Passion drive our decisions to success

Our products and product candidates combine our TransCon technologies with clinically validated parent drugs or pathways, with the goal of optimizing safety, efficacy, tolerability and convenience.

We apply these technologies using our algorithm with the goal of creating product candidates with the potential to be best-in-class. Using this approach, we plan to expand our pipeline with Endocrinology Rare Disease product opportunities in large addressable markets. In addition, our vision is to pursue TransCon product opportunities in >\$5B indications in other therapeutic areas and maximize value creation of these product opportunities through collaboration with therapeutic area market leaders. We believe our approach to product innovation may reduce the risks associated with traditional drug development.

### Ascendis Algorithm for Product Innovation



When we apply our TransCon technologies to clinically validated parent drugs or pathways, we may benefit from established clinical safety and efficacy data, which we believe increases the probability of success compared to traditional drug development. As illustrated above, our algorithm for product innovation focuses on identifying indications that have an unmet medical need, have a clinically validated parent drug or pathway, are suitable to our TransCon technologies, have potential for creating a clearly differentiated product, have a potential established development pathway, and have the potential to address a large market.

### Program Summaries

We currently have two marketed products and a diversified portfolio of four product candidates in clinical development in the areas of Endocrinology Rare Disease and Oncology, and we are working to apply our TransCon technology platform in additional therapeutic areas such as the glucagon-like peptide 1 (“GLP-1”) class, where we believe we have designed a potentially best-in-class, once-monthly program.

- *SKYTROFA* – Our first marketed product was SKYTROFA® (lonapegsomatropin-tcgd), developed as TransCon Growth Hormone (“TransCon hGH”), which has received regulatory approval in the United States for the treatment of pediatric patients one year and older who weigh at least 11.5 kg and have growth failure due to inadequate secretion of endogenous growth hormone, also known as growth hormone deficiency (“GHD”). TransCon hGH has been commercially available for prescription in the United States under its brand name SKYTROFA (lonapegsomatropin-tcgd) since October 2021. In addition, TransCon hGH was granted marketing authorization in the European Union (“EU”), Norway, Iceland, Lichtenstein and Great Britain (covering England, Wales, Scotland) as SKYTROFA (lonapegsomatropin), a once-weekly subcutaneous injection for the treatment of children and adolescents ages 3 to 18 years with growth failure due to insufficient secretion of endogenous growth hormone. SKYTROFA has been commercially available for prescription in Germany since September 2023.



- **YORVIPATH** – Our second marketed product is YORVIPATH<sup>®</sup> (palopegteriparatide), developed as TransCon PTH. In the EU, Norway, Iceland, Lichtenstein and Great Britain (covering England, Wales, Scotland), YORVIPATH has been granted marketing authorization as a once-daily subcutaneous injection for the treatment of adults with chronic hypoparathyroidism. YORVIPATH has been commercially available for prescription in Germany and Austria since January 2024. In the U.S., TransCon PTH received regulatory approval from the U.S. Food and Drug Administration (“FDA”) for the treatment of hypoparathyroidism in adults in August 2024 under the brand name YORVIPATH. We are completing manufacturing of commercial product for the U.S. market and anticipate initial supply will be available in the first quarter of 2025. We are in dialogue with the FDA about commercialization of existing manufactured products, which if agreed, could be introduced in the U.S. in the fourth quarter of 2024.
- **Endocrinology Rare Disease Pipeline** – We are further developing two product candidates in our Endocrinology Rare Disease portfolio spanning multiple indications and geographies. These product candidates are TransCon hGH for adult GHD and Turner syndrome and TransCon CNP (navepegritide) for infants and children with achondroplasia. Through our strategic collaboration, Teijin Limited is developing and, if approved, plans to commercialize TransCon hGH, TransCon PTH, and TransCon CNP for endocrinology rare disease in Japan. In addition, through our strategic investment, VISEN Pharmaceuticals (“VISEN”) is developing and, if approved, plans to commercialize TransCon hGH, TransCon PTH, and TransCon CNP for endocrinology rare diseases in Greater China.
- **Oncology Pipeline** – In Oncology, we are leveraging our TransCon technologies with the goal of enhancing the anti-tumor effects of clinically-validated parent drugs and pathways and to provide sustained modulation of tumor microenvironments and activate cytotoxic immune cells. We have initiated clinical development of two product candidates: TransCon TLR7/8 Agonist, an investigational, long-acting prodrug of resiquimod, a small molecule agonist of Toll-like receptors (“TLR”) 7 and 8 for intratumoral delivery, and TransCon IL-2 b/g (onvapegleukin alfa) for systemic delivery, which is designed for prolonged exposure to an IL-2 variant that selectively activates the IL-2 b/g, with minimal binding to IL-2R $\alpha$ . Our clinical development program for these product candidates also includes evaluation of each of them as a potential combination therapy.

### TransCon Products and Product Candidates Pipeline

Other than the rights we have granted to VISEN, Teijin Limited, and Eyconis as noted in this report, we hold worldwide rights to our TransCon technologies and, other than our royalty financing arrangements with Royalty Pharma as noted in this report, we owe no third-party royalty or milestone payment obligations with respect to our TransCon technologies, TransCon hGH, TransCon PTH or any of our other product candidates. The following chart lists our approved products and product candidates.

Endocrinology Rare Diseases	Indication	Status	Region	
<b>Approved Products</b>	SKYTROFA <sup>®</sup>	• Pediatric Growth Hormone Deficiency (GHD) <sup>1,2</sup>	Approved	• US, EU, Norway, Iceland, Lichtenstein and Great Britain (covering England, Wales, Scotland)
	YORVIPATH <sup>®</sup>	• Hypoparathyroidism in adults <sup>3,4</sup>	Approved	• US, EU, Norway, Iceland, Lichtenstein and Great Britain (covering England, Wales, Scotland)
<b>Independent Product Candidate (lead indication)</b>	TransCon CNP	• Achondroplasia (children ages 2–11)	Pivotal <sup>5</sup>	• Multinational
<b>Label Expansion</b>	TransCon hGH	• Adult Growth Hormone Deficiency	Phase 3 <sup>6</sup>	• Multinational
		• Turner Syndrome	Phase 2 <sup>7</sup>	• US
	TransCon CNP	• Achondroplasia (infants)	Phase 2 <sup>8</sup>	• Multinational
	TransCon CNP + TransCon hGH	• Achondroplasia (children ages 2–11)	Phase 2 <sup>9</sup>	• Multinational
<b>Partner Programs</b>	TransCon hGH	• Pediatric GHD	BLA submitted <sup>10</sup>	• China
	TransCon hGH	• Pediatric GHD	Phase 3 <sup>11</sup>	• Japan
	TransCon PTH	• Hypoparathyroidism in adults	Phase 3 <sup>12,13</sup>	• China, Japan
	TransCon CNP	• Achondroplasia	Phase 2 <sup>14</sup>	• China
<b>Oncology</b>				
<b>Independent Product Candidate</b>	TransCon TLR7/8 Agonist	• Various tumor types	Phase 2 <sup>15,16</sup>	• Multinational
	TransCon IL-2 $\beta/\gamma$	• Various tumor types	Phase 2 <sup>16,17</sup>	• Multinational

1. In the U.S., SKYTROFA is indicated for the treatment of pediatric patients 1 year and older who weigh at least 11.5 kg and have growth failure due to inadequate secretion of endogenous growth hormone.
2. In the EU, SKYTROFA is indicated for growth failure in children and adolescents aged from 3 years up to 18 years due to insufficient endogenous growth hormone secretion.
3. In the U.S., YORVIPATH is indicated for the treatment of hypoparathyroidism in adults.

4. *In the EU, the therapeutic indication for YORVIPATH is a parathyroid hormone replacement therapy indicated for the treatment of adults with chronic hypoparathyroidism.*
5. *Pivotal ApproaCH Trial (NCT05598320).*
6. *foresiGHt Trial (NCT05171855).*
7. *New InsiGHTS Trial (NCT05690386).*
8. *reACHin Trial (NCT06079398).*
9. *COACH Trial (NCT06433557).*
10. *VISEN Pharmaceuticals' Phase 3 trial.*
11. *Japanese riGHt Trial.*
12. *PaTHway China.*
13. *PaTHway Japan.*
14. *ACcomplish China.*
15. *transcendIT-101 Trial (NCT04799054).*
16. *BelieveIT-201 Trial (NCT05980598).*
17. *IL-Believe Trial (NCT05081609).*

We maintain an intellectual property portfolio comprising over 300 issued patents and over 550 patent applications as of December 31, 2023, which includes patents and patent applications applicable to our products and product candidates with claims directed to composition of matter, process, formulation and/or methods-of-use for our products and product candidates, including a product-specific device and core TransCon technologies. Other than the rights we have granted to VISEN, Teijin Limited, and Eyconis as noted in this report, we hold worldwide rights to our TransCon technologies and, other than our royalty financing arrangements with Royalty Pharma as noted in this report, we owe no third-party royalty or milestone payment obligations with respect to our TransCon technologies, TransCon hGH, TransCon PTH, or any of our other product candidates. While our TransCon prodrugs may incorporate already approved parent drugs, TransCon hGH, TransCon PTH and each of our other product candidates are new molecular entities and therefore eligible to be granted new intellectual property rights, including new composition of matter patents.

### **Global Commercialization Strategy**

We are establishing a global presence to commercialize TransCon product and product candidates, if approved, to address patients' unmet medical needs.

In the U.S., we have established a multi-faceted organization to support the ongoing commercialization of SKYTROFA, which will also serve as the foundation for future Endocrinology Rare Disease product launches in the U.S., including YORVIPATH.

In Europe, we are expanding our presence by building integrated organizations in select countries, which we call Europe Direct, beginning with Germany, where we have launched SKYTROFA and YORVIPATH. We are establishing commercial infrastructure in other Europe Direct country clusters, including DACH (Germany, Austria, Switzerland), France & BeNeLux (Belgium, the Netherlands, and Luxembourg), Iberia (Portugal and Spain), Italy, Nordics (Denmark, Norway, Sweden, Iceland, Finland), and the United Kingdom & Ireland.

Beyond the U.S. and Europe Direct, we are expanding global reach for our Endocrinology Rare Disease programs through exclusive distribution agreements with geographic market leaders, which we call International Markets. As of June 30, 2024, we have established five such regional agreements:

- Specialised Therapeutics Asia Pte Ltd. (Australia, New Zealand, Singapore, Malaysia, Brunei, Thailand, and Vietnam)
- Er-Kim İlac Sanayi ve Ticaret A.S (Central & Eastern Europe and Turkey)
- Vector Pharma FZCO (Saudi Arabia, United Arab Emirates, Kuwait, Oman, Qatar, and Bahrain)
- Neopharm (Israel) 1996 Ltd (Israel)
- Pendopharm, a division of Pharmascience Inc. (Canada)

Finally, we are making our products commercially available in select markets through exclusive license agreements with partners with local development and commercialization expertise and infrastructure. In China, VISEN has exclusive license rights to develop and commercialize TransCon hGH, TransCon PTH, and TransCon CNP. In Japan, Teijin Limited has exclusive license rights to develop and commercialize TransCon hGH, TransCon PTH, and TransCon CNP.

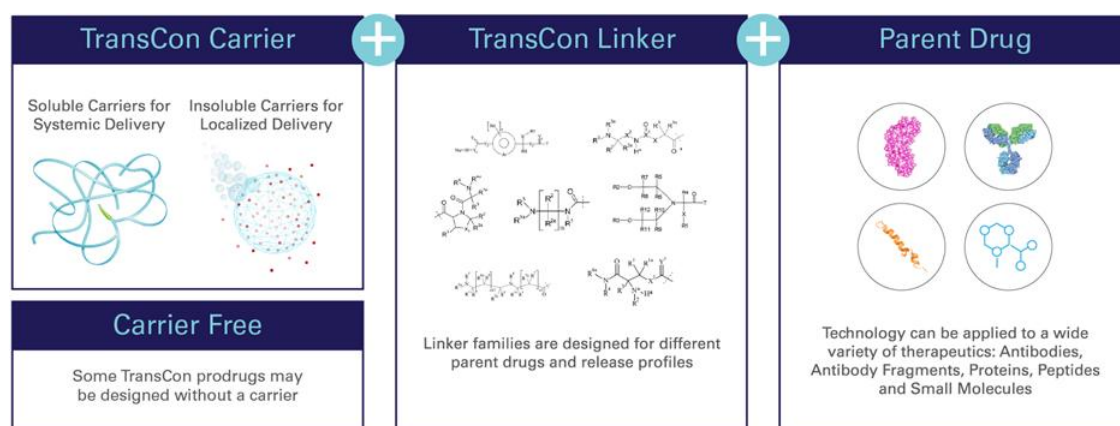
## TransCon Technologies

### Overview

Our TransCon technologies are designed to combine the benefits of conventional prodrug and sustained release technologies to solve the fundamental limitations seen in other approaches to extending duration of a drug's action in the body, with the goal of developing highly differentiated product candidates based on efficacy, safety, tolerability and convenience. In addition to retaining the original mode of action of the parent drug and potentially supporting dosing frequency from daily up to six months or more, we believe that predictable release over time can improve treatment safety and efficacy, increase the likelihood of clinical development success, and provide intellectual property benefits.

TransCon molecules can have up to three components: a parent drug, an inert carrier that protects it, and a linker that temporarily binds the two. When bound, the carrier inactivates and shields the parent drug from clearance. When injected into the body, physiologic pH and temperature conditions initiate the release of the active, unmodified parent drug in a predictable release manner.

Depending upon the type of TransCon carrier we employ, we can design our TransCon prodrugs for sustained localized or systemic delivery.

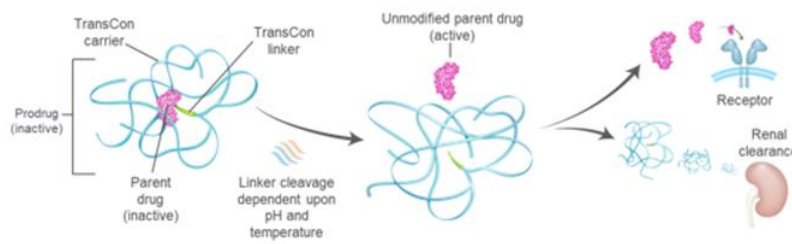


### TransCon Technology Components

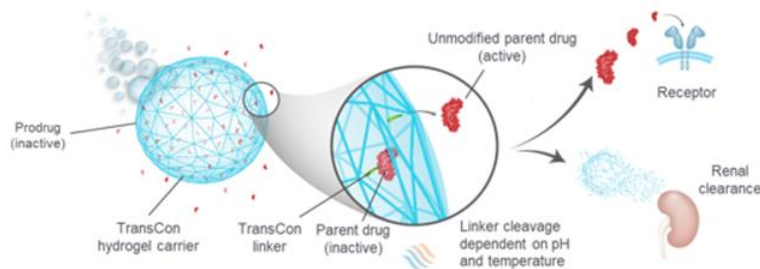
#### TransCon Carriers

Our TransCon technologies incorporate two carrier platforms that can be used to provide sustained localized or systemic drug exposure. These biocompatible carrier platforms include our TransCon systemic carriers and TransCon localized carriers (self-eliminating hydrogels). Our carriers inactivate and protect the drug through a shielding effect, which may prevent rapid excretion and degradation of the parent drug and enable benefits that include improved injection site tolerability, reduced systemic adverse effects, and low immunogenicity.

- **Systemic** – Our TransCon systemic carriers are used to provide systemic drug exposure and are based on soluble compounds such as methoxypolyethylene glycol (“mPEG”) or other natural or synthetic polymers. Prodrugs created using our systemic carriers are readily absorbed into the bloodstream after administration, thus minimizing exposure of the subcutaneous tissue to active drug, which we believe may improve injection site tolerability. TransCon hGH, TransCon PTH, and TransCon CNP utilize mPEG as a carrier molecule. mPEG is widely used to improve the pharmacokinetic or pharmacodynamic properties of marketed therapeutics. Below is an illustration of our systemic carrier:



- Localized** – Our TransCon localized carriers include TransCon hydrogels based on PEG, hyaluronic acid, or other biopolymers. TransCon hydrogel is designed to self-eliminate to soluble, biocompatible molecules after the drug payload has been released. When applied for localized delivery, the TransCon hydrogel enables the release of a parent drug at high local concentrations within the target area while minimizing systemic exposure. We believe this may widen the therapeutic window for parent drugs that suffer from significant systemic side effects and toxicities, facilitating the development of highly efficacious product candidates with improved safety and tolerability profiles. Below is an illustration of our hydrogel carrier:



- In 2023, we announced the development of a novel TransCon prolongation technology. This new TransCon technology may support expansion of TransCon technology into new therapeutic areas.

#### TransCon Linkers

Our reversible TransCon linkers are designed to enable the transient conjugation of a broad range of therapeutics, including proteins, peptides and small molecules, to our TransCon carriers. We have a large library of TransCon linkers that may be applicable to various types of parent drugs, and that can be tailored to potentially achieve half-life extension enabling daily, weekly, monthly, and half-yearly dosing and to customize the potential pharmacokinetic profile for each individual product candidate with the goal of optimizing the potential therapeutic effect. TransCon linkers are self-cleaving through a process called intra-molecular assisted cleavage, which causes the linker to release the unmodified parent drug. We can tailor the release properties of the linker to a given therapeutic indication and parent drug by modifying the linker structures. We believe the self-cleaving process of our linker avoids many of the shortcomings of conventional prodrug technologies, which often depend on metabolic processes, such as enzymatic degradation, to convert the prodrug into the active drug. The rate of metabolic conversion of prodrugs in these types of processes may differ between patients, and even within different tissues in the same patient. As a result, conventional prodrugs do not always offer predictable release of the parent drug. Our TransCon linkers are designed to predictably release an unmodified active parent drug at predetermined rates governed by physiological pH and temperature conditions, which are tightly regulated in the body. Consequently, we believe we can design our prodrugs to release the unmodified parent drug at predictable rates.

## *Parent Drugs*

Our TransCon technologies are applicable across a broad range of therapeutic classes and are currently used to create potentially best-in-class long-acting product candidates based on proteins, peptides and small molecules. By primarily focusing on biological targets that have been clinically validated, we can leverage available knowledge regarding a target's activity. Based on this selective approach, we know what drug levels must be maintained in the body for optimal efficacy and safety, and we can design the release half-life and dosing frequency of our TransCon prodrugs to maintain these levels to achieve the desired pharmacological effect. We move a product candidate into development after it demonstrates the desired profile in non-clinical models.

Furthermore, based on the established translational relationships between preclinical animal models and clinical efficacy, we believe experimental results generated in animal models are highly predictive of clinical results and reduce the development risk for our TransCon prodrugs. This strategy is designed to reduce risk and increase productivity.

This approach has enabled us to develop two approved products and generate a pipeline of product candidates designed to address significant unmet medical needs and to become potential sources of significant revenue for our company. Because our TransCon technologies leverage clinically validated parent drugs or pathways, we believe we may benefit from a higher development and regulatory success rate compared to development of drug compounds without established biology.

## **TransCon Products – Endocrinology Rare Disease**

### ***TransCon Growth Hormone (hGH)***

#### *Market Opportunity for Recombinant Human Growth Hormone*

Growth hormone deficiency is a serious orphan disease that affects both children and adults. Children with GHD are characterized by short stature, metabolic and cardiovascular abnormalities, cognitive deficiencies, and poor quality of life. GHD in adults is associated with increased adiposity, or fat mass, as well as psychiatric-cognitive, cardiovascular, muscular, metabolic and skeletal abnormalities. In childhood and adolescence, growth hormone plays an essential role in normal longitudinal growth, muscle and bone strength, and distribution of body fat. In adults, growth hormone contributes to body composition, cardiovascular function, and bone health. The current standard of care for GHD has been daily subcutaneous injections of somatropin, a recombinant human growth hormone (“hGH”). These daily hGH therapies have been shown to be safe and well-tolerated.

In both therapy-compliant children and adults with GHD, daily subcutaneous injections of hGH result in improved body composition parameters, bone density, cardiovascular outcomes and quality of life. Growth hormone-deficient children who are fully adherent to their daily hGH treatment regimen may achieve a height in adulthood that is comparable to that of their family members and national norms.

Despite the demonstrated benefits of daily hGH therapy, many GHD patients are not adequately treated, and adherence continues to be a challenge, as reported in a 2021 paper published by Kaplowitz et al. (Kaplowitz P, Manjelievskaia J, Lopez-Gonzalez L, et al. Economic burden of growth hormone deficiency in a US pediatric population. *J Manag Care Spec Pharm.* 2021; 27(8):1118-1128). The observational retrospective cohort analysis utilized administrative claims data from two databases on over 20,000 pediatric patients diagnosed with GHD. Approximately 68% of commercial patients and approximately 63% of Medicaid patients received daily growth hormone treatment, whereas approximately 32% of commercial patients and approximately 37% of Medicaid patients were untreated. In addition, mean adherence as measured by proportions of days covered which is defined as the number of days covered by any daily growth hormone prescription during the follow-up period was approximately 60% in the commercial cohort and approximately 50% in the Medicaid cohort. Only 32% of commercial and 18% of Medicaid patients reported adherence rates greater than 80%.

For adult patients with GHD, underdiagnosis and undertreatment are also a concern. Untreated adult GHD (“AGHD”) patients can experience reduced quality of life and increased risk of morbidity and mortality. A retrospective cohort study presented at ENDO 2023 analyzed an electronics health records database and selected adult patients with suspected AGHD. Of the 51,588 patients with suspected AGHD, fewer than 4% were treated with growth hormone.

Since the introduction of hGH in 1981, a number of the world’s largest pharmaceutical companies have developed and marketed daily-administered hGH products. All currently marketed daily hGH products in the United States – Norditropin® (Novo Nordisk A/S), Humatrope® (Eli Lilly and Company), Genotropin® (Pfizer Inc.), Zomacton® (Ferring Pharmaceuticals, Inc.) and Omnitrope® (Sandoz GmbH) – contain unmodified somatropin (hGH) and are administered by subcutaneous injections. The global market for daily hGH products is largely composed of products from Novo Nordisk, Pfizer, Eli Lilly, Sandoz, and Merck KGaA, which together account for most of the global market share.

Primary indications for hGH in children are GHD, idiopathic short stature, chronic kidney disease, Prader-Willi syndrome, small for gestational age, and Turner syndrome. In adults, primary indications for hGH include GHD and AIDS-induced weight loss. We estimate pediatric indications comprise up to 90% of the current total hGH market, of which approximately half is for pediatric GHD.

#### *Competitive Landscape for Long-Acting Growth Hormone Therapies*

Since the 1990s, the pharmaceutical industry has employed various approaches to develop long-acting growth hormone products to reduce the burden of daily injections on patients and increase patient compliance with the dosing regimen. These approaches generally fall into two categories: unmodified somatropin (hGH) and permanent modification of growth hormone:

- **Unmodified somatropin (hGH):** Two long-acting growth hormone products using encapsulation technologies previously received regulatory approval in the U.S. and Europe but were subsequently discontinued due to commercial challenges. These include Nutropin Depot®, formerly marketed by Genentech, and Somatropin Biopartners, developed by LG Life Sciences and Biopartners GmbH. Nutropin Depot was approved in 1999 and later withdrawn; Somatropin Biopartners (LB03002), was approved by the European Medicines Agency (“EMA”) in 2013, and later withdrawn. We believe that the lack of market acceptance was a result of the various safety and tolerability issues that tend to arise with encapsulation technologies.
- **Permanent modification of growth hormone:** Modification technologies prolong activity in the body by creating analogs of growth hormone through permanent modification of the growth hormone molecule. This modification may alter the molecular size and interaction with the growth hormone receptor and/or change the natural association affinity to endogenous proteins, as well as the distribution in the body. These changes may alter and reduce the efficacy of these drugs compared to unmodified daily somatropin (hGH) and may also negatively impact the drug’s safety.

Novo Nordisk received regulatory approval in various countries and regions including the U.S., Japan, and EU for once-weekly somapacitan (SOGROYA®) for replacement of endogenous growth hormone in adult patients with GHD and pediatric patients with GHD.

Pfizer (in collaboration with OPKO Health Inc.) received regulatory approval of once-weekly somatrogen (NGENLA) in various countries and regions including the U.S., Japan, and EU for pediatric GHD.

A permanently PEGylated long-acting growth hormone developed by GeneScience Pharmaceuticals Co., Ltd. (Jintrolong®) is available in China and the Somatropin Biopartners product (LB03002) is available in Korea. Other experimental growth hormone therapies based on permanent modification are in different stages of clinical development by various companies, including Genexine Inc., I-MAB, and JCR Pharmaceuticals Co., Ltd.

#### *Our Solution: TransCon hGH*

TransCon hGH is a prodrug composed of somatropin that is transiently bound to a carrier by a proprietary linker. TransCon hGH is administered once weekly and is designed to maintain the same mode of action as daily therapies by providing sustained release of active, unmodified somatropin, the same recombinant growth hormone molecule used in the daily hGH therapies that are the current standard of care.

### ***TransCon Growth Hormone (hGH) for pediatric GHD***

TransCon hGH, marketed under the brand name SKYTROFA<sup>®</sup> (lonapegsomatropin-tcgd), received regulatory approval in the U.S. for the treatment of pediatric patients one year and older who weigh at least 11.5 kg and have growth failure due to inadequate secretion of endogenous growth hormone, also known as GHD. SKYTROFA has been commercially available for prescription in the United States since October 2021. In the EU, Norway, Iceland, Lichtenstein and Great Britain (covering England, Wales, Scotland), we received marketing authorization for TransCon hGH – known by its brand name SKYTROFA (lonapegsomatropin) – as a once-weekly subcutaneous injection for the treatment of children and adolescents ages 3 to 18 years with growth failure due to insufficient secretion of endogenous growth hormone. SKYTROFA has been commercially available for prescription in Germany since September 2023.

In September 2023, we announced topline results from the completed enliGHten Trial, an open-label extension trial evaluating the long-term safety and efficacy of TransCon hGH as a once-weekly treatment for children and adolescents with growth hormone deficiency. The enliGHten Trial enrolled 298 participants (mean age 10.3 years) from the Phase 3 heiGHt Trial of treatment-naïve pediatric GHD patients and the Phase 3 fliGHt Trial of pediatric GHD patients switching from daily somatropin treatment. Patients in these trials received a total of up to 6 years of treatment with TransCon hGH. At the time of the enliGHten Trial closure, 81 participants were designated as treatment completers, based on their physician’s determination that treatment for pediatric GHD was no longer required. Of these treatment completers, 59% met or exceeded their average parental height standard deviation score (“SDS”), with mean TransCon hGH treatment duration of 3.2 years.

### **Clinical Trial of TransCon hGH in Japanese Pediatric GHD**

In our ongoing Phase 3 riGHt Trial, we are evaluating TransCon hGH as a treatment in Japanese children with GHD. The primary objective of the riGHt Trial is to evaluate and compare the annualized height velocity of approximately 40 Japanese prepubertal children with GHD treated with once-weekly TransCon hGH to that of children treated with a commercially available daily hGH formulation at 52 weeks. Enrollment in the riGHt trial was completed during the fourth quarter of 2023.

### ***Proprietary Auto-injector***

SKYTROFA includes the SKYTROFA<sup>®</sup> Auto-Injector and cartridges. The auto-injector provides for room temperature storage, includes an empty-all design, and is expected to last for at least four years. The device enables a single, low-volume injection of less than 0.6 mL for the majority of patients with a thin, 31-gauge needle that is only 4 millimeters in length, which is comparable to needles used to administer daily hGH. We are also working on strategies that will enable the auto-injector to integrate with the digital healthcare system, including Bluetooth connectivity features to allow for easy tracking of dosing adherence over time.



*Figure: Our state-of-the-art auto-injector is designed to improve treatment compliance for children with GHD.*

## **TransCon Product Candidates – Endocrinology Rare Diseases**

### ***TransCon Growth Hormone (hGH) for Other Indications***

#### *Clinical Development in Adults*

In December 2023, we announced positive topline results from foresiGHt, a Phase 3 randomized, parallel-arm, placebo-controlled (double-blind) and active-controlled (open-label) trial to compare the efficacy and safety of TransCon hGH with placebo and daily hGH in adults with GHD. The trial aims to evaluate the metabolic benefits of TransCon hGH in adults, with the primary objective to evaluate change in trunk fat percentage.

The foresiGHt Trial evaluated 259 adults with GHD aged 23 to 80 years old, randomized 1:1:1, titrated to receive a target fixed dose of TransCon hGH, placebo, or daily hGH based on age and oral estrogen intake, with approximately equivalent hGH mg/week for TransCon hGH and daily hGH.

- TransCon hGH demonstrated superiority on its primary efficacy endpoint at Week 38:
  - o Change from baseline in trunk percent fat as measured by dual x-ray absorptiometry (TransCon hGH -1.67% vs. placebo +0.37%, LS mean difference = -2.04%,  $p < 0.0001$ )
- TransCon hGH demonstrated superiority on its key secondary efficacy endpoints at Week 38:
  - o Change from baseline in total body lean mass (TransCon hGH +1.60 kg vs placebo -0.10 kg, LS mean difference = 1.70 kg,  $p < 0.0001$ )
  - o Change from baseline in trunk fat mass (TransCon hGH -0.48 kg vs placebo +0.22 kg, LS mean difference = -0.70 kg,  $p = 0.0053$ )
- Exploratory post-hoc analysis at Week 38 demonstrated comparable treatment effect of TransCon hGH and daily hGH on target tissues. For patients with average IGF-1 SDS levels  $\leq 1.75$  at Week 38:
  - o Change from baseline in trunk percent fat (TransCon hGH -2.42% vs. daily hGH -2.59%)
  - o Change from baseline in total body lean mass (TransCon hGH +1.70 kg vs daily hGH +1.37 kg)
  - o Change from baseline in trunk fat mass (TransCon hGH -0.90 kg vs daily hGH -0.94 kg)
- TransCon hGH was generally safe and well tolerated, with no discontinuations related to study drug and with comparable safety and tolerability to daily hGH.

We plan to submit a supplemental Biologics License Application (“BLA”) to the FDA for adult GHD in the third quarter of 2024.

#### *Other Development Plans*

In June 2022, we initiated the Phase 2 New InsiGHTS Trial in the U.S. to evaluate TransCon hGH in Turner syndrome. In this trial, we are evaluating higher doses of TransCon hGH and daily hGH for Turner syndrome compared to doses for pediatric or adult GHD. Topline results from New InsiGHTS are expected in the fourth quarter of 2024. In addition, we are investigating other potential indications for TransCon hGH where we believe a long-acting hGH therapy may offer benefits to patients with rare growth disorders, including in combination with our TransCon CNP product candidate.

### ***TransCon PTH***

#### *Market Opportunity in Hypoparathyroidism*

Hypoparathyroidism is a rare endocrine disease characterized by insufficient levels of parathyroid hormone (“PTH”). Most patients with hypoparathyroidism develop the condition following damage to or accidental removal of the parathyroid glands during thyroid surgery. Post-surgical hypoparathyroidism accounts for the majority of cases (70-80%); other etiologies include autoimmune disorders, genetic disorders such as autosomal dominant hypocalcemia type 1, and idiopathic causes. Conventional therapy with oral calcium and active vitamin D (also called calcitriol) does not effectively address the short-term symptoms, long-term complications, or quality-of-life impacts of hypoparathyroidism.



Short-term symptoms include weakness, severe muscle cramps (tetany), abnormal sensations such as tingling, burning and numbness (paresthesia), memory loss, impaired judgment, and headache. Patients often experience decreased quality of life, and, over the long term, prolonged use of conventional therapy may increase risk of major complications, such as calcium deposits in the brain, blood vessels, eye, and other soft tissues. According to a recent systematic literature review, chronic hypoparathyroidism treated with conventional therapy is associated with higher rates of renal complications compared to the general population, such as nephrolithiasis (up to 36%), nephrocalcinosis (up to 38%), and chronic kidney disease (up to 41%).

Hypoparathyroidism also poses a high burden on the healthcare system despite current conventional therapy. For example, one survey of 374 patients showed that 72% experienced more than ten symptoms in the preceding twelve months, with symptoms experienced for a mean of  $13 \pm 9$  hours a day. Other studies showed that 79% of hypoparathyroidism cases require hospitalizations and that patients with the disease have a four-fold increase in the risk of renal disease compared to healthy controls. Patients often experience decreased quality of life. We conducted a survey of 42 patients which found that 100% of patients reported negative psychological impacts, interference with daily life and impact on physical functioning from hypoparathyroidism, and that 76% were either no longer able to work or experienced interference with work productivity.

The 2022 Guidelines from the Second International Workshop addressing the prevention, diagnosis, and management of hypoparathyroidism was published in September 2022 in the Journal of Bone and Mineral Research and authored by leading clinicians from North America, Europe, and Asia. The authors suggest consideration of PTH replacement therapy in patients whose hypoparathyroidism is inadequately controlled with conventional therapy. Inadequate control is considered to be any one of the following: symptomatic hypocalcemia, hyperphosphatemia, renal insufficiency, hypercalciuria, or poor quality of life. In addition, the guideline indicates that individuals with poor compliance, malabsorption or who are intolerant of large doses of calcium and active vitamin D may also benefit from PTH replacement therapy. Based on this current guideline, we believe PTH replacement therapy could be applicable to most patients with hypoparathyroidism.

In 2015, NATPARA<sup>®</sup> (parathyroid hormone) for injection was approved in the U.S. for once-daily subcutaneous injection as an adjunct to vitamin D and calcium in patients with hypoparathyroidism. NATPARA was voluntarily recalled in September 2019 in the U.S. and is now only available to a limited number of patients through a Special Use Program offered by its manufacturer, Takeda. In October 2022, Takeda announced that it will discontinue manufacturing NATPARA/NATPAR globally by the end of 2024.

We are also aware of several academic groups and companies working on making longer-acting agonists of the PTH receptor. In addition, other companies and groups are developing or commercializing therapies for hypoparathyroidism, including Calcilytix (a BridgeBio company), Entera Bio, Extend Biosciences, Massachusetts General Hospital, Amolyt Pharma (acquired by AstraZeneca), and MBX Biosciences.

Teriparatide, PTH (1-34), approved since 2002 for the treatment of osteoporosis, has sometimes been used for treatment of hypoparathyroidism using multiple daily injections, despite not being approved for this indication. Clinical research conducted by the U.S. National Institutes of Health in subjects receiving continuous exposure to PTH (1-34), administered by an infusion pump, demonstrated simultaneous normalization of sCa and urinary calcium, as well as normalization of bone turnover.

We estimate hypoparathyroidism affects over 250,000 patients in the U.S. and Europe. In the U.S., we estimate hypoparathyroidism affects approximately 70,000 to 90,000 patients, including 4,000 to 5,000 patients who we estimate have previously been treated with PTH therapy. In Germany, we estimate hypoparathyroidism affects approximately 70,000 patients. Outside of Germany, we estimate hypoparathyroidism affects over 100,000 patients in the rest of Europe.

#### *Our Solution: TransCon PTH*

TransCon PTH (palopegteriparatide) is a prodrug of PTH (1-34) that is designed to be dosed once-daily to achieve and maintain a steady concentration of PTH in the bloodstream within the normal range. TransCon PTH is designed to provide PTH in the physiological range for 24 hours per day, thereby more fully addressing aspects of the disease, including normalizing serum and urinary calcium and serum phosphate levels.

By providing steady levels of PTH in the physiological range, we believe TransCon PTH can address the fundamental limitations of PTH therapies with short half-life molecules and become a highly differentiated therapy for hypoparathyroidism.

### **TransCon Parathyroid Hormone (PTH) for adults with hypoparathyroidism**

In November 2023, TransCon PTH received regulatory approval in the EU and European Economic Area and is marketed as YORVIPATH® (palopegteriparatide), a parathyroid hormone replacement therapy indicated for the treatment of adults with chronic hypoparathyroidism. In April 2024, TransCon PTH received regulatory approval in Great Britain as a PTH replacement therapy indicated for the treatment of adults with chronic hypoparathyroidism. In August 2024, the U.S. FDA approved TransCon PTH for the treatment of hypoparathyroidism in adults. We are completing manufacturing of commercial product for the U.S. market and anticipate initial supply will be available in the first quarter of 2025. We are in dialogue with the FDA about commercialization of existing manufactured products, which if agreed, could be introduced in the U.S. in the fourth quarter of 2024.

In January 2024, we announced that YORVIPATH is commercially available in Germany and Austria, and we began shipping to customers in February. In April 2024, we announced that the United Kingdom’s Medicines & Healthcare products Regulatory Agency granted YORVIPATH orphan drug status.

#### **Clinical Development of TransCon PTH for Adult Hypoparathyroidism**

Our ongoing Phase 3 PaTHway Trial, Phase 3 PaTHway Japan Trial, and Phase 2 PaTH Forward Trial continue in the open-label extension portion to collect long-term data.

In May 2024, we announced 2-year results from a post-hoc analysis of the our Phase 3 PaTHway Trial demonstrating significant and sustained improvements in renal function in adults with chronic hypoparathyroidism treated with TransCon PTH. The post-hoc analysis examined the impact of treatment with TransCon PTH on renal function using estimated glomerular filtration rate (“eGFR”) through Week 104 (n=76) of PaTHway, a Phase 3 double-blind, placebo-controlled trial of 82 dosed adults with chronic hypoparathyroidism randomized 3:1 (TransCon PTH:placebo; both arms initially co-administered with conventional therapy of active vitamin D and oral calcium), with a 26-week blinded period followed by an ongoing 156-week open-label extension period. Across both treatment arms, TransCon PTH treatment resulted in a mean eGFR increase of 8.9 mL/min/1.73m<sup>2</sup> (p<0.0001) from baseline at Week 52, sustained at Week 104 with a mean change from baseline of 9.0 mL/min/1.73m<sup>2</sup> (p<0.0001). Treatment was generally well-tolerated, with no new safety signals.

<b>eGFR* Change from Baseline by Study Arm</b>							
<b>Study Arm</b>	<b>Baseline</b>	<b>Week 26</b>		<b>Week 52</b>		<b>Week 104</b>	
	<b>eGFR (mL/min/1.73m<sup>2</sup>)</b>	<b>N</b>	<b>Mean (p value)</b>	<b>N</b>	<b>Mean (p value)</b>	<b>N</b>	<b>Mean (p value)</b>
TransCon PTH / TransCon PTH	eGFR < 60	19	+11.4 (p=0.0002)	19	+11.5 (p=0.0003)	18	+13.4 (p<0.0001)
	eGFR ≥ 60	41	+6.3 (p=0.0002)	40	+8.6 (p<0.0001)	40	+6.9 (p<0.0001)
	All	60	+7.9 (p<0.0001)	59	+9.3 (p<0.0001)	58	+8.9 (p<0.0001)
Placebo (first 26 weeks) / TransCon PTH**	eGFR < 60	4	+0.1 (p=0.9877)	4	+11.7 (p=0.0018)	4	+15.6 (p=0.0067)
	eGFR ≥ 60	15	-2.4 (p=0.3280)	15	+6.5 (p=0.0199)	14	+7.6 (p=0.0121)
	All	19	-1.9 (p=0.3468)	19	+7.6 (p=0.0014)	18	+9.4 (p=0.0006)

\*eGFR (an assessment of kidney filtering capacity) was calculated by the trial’s central lab using the Modification of Diet in Renal Disease Study Group (MDRD) equation (Levey, *Ann Intern Med* 2006). An eGFR level <60 mL/min/1.73m<sup>2</sup> is considered the threshold for impaired kidney function.

\*\*Patients in the placebo arm switched to TransCon PTH following the Week 26 visit.

TransCon PTH treatment was associated with clinically meaningful increases ( $\geq 5$  mL/min/1.73 m<sup>2</sup>) in eGFR within 26 weeks that were sustained through Week 104 of PaTHway:

Proportion of Participants (%) with $\geq 5$ and $\geq 10$ mL/min/1.73 m <sup>2</sup> Increases in eGFR from Baseline through Week 104*						
eGFR Change from Baseline	All Participants					
	TransCon PTH / TransCon PTH (n=61)			Placebo (first 26 weeks) / TransCon PTH** (n=21)		
	Week 26	Week 52	Week 104	Week 26	Week 52	Week 104
	PTH	PTH	PTH	Placebo	Switch to PTH	Switch to PTH
$\geq 5$ mL/min/1.73 m <sup>2</sup>	57%	64%	61%	24%	52%	62%
$\geq 10$ mL/min/1.73 m <sup>2</sup>	43%	43%	46%	10%	29%	38%

eGFR Change from Baseline	Participants with Baseline eGFR < 60 mL/min/1.73 m <sup>2</sup>					
	TransCon PTH / TransCon PTH (n=19)			Placebo (first 26 weeks) / TransCon PTH** (n=4)		
	Week 26	Week 52	Week 104	Week 26	Week 52	Week 104
	PTH	PTH	PTH	Placebo	Switch to PTH	Switch to PTH
$\geq 5$ mL/min/1.73 m <sup>2</sup>	74%	68%	74%	25%	100%	100%
$\geq 10$ mL/min/1.73 m <sup>2</sup>	47%	42%	53%	0%	75%	75%

\*Percentages were calculated based on all participants. Patients who did not have an eGFR assessment at the visit were still included in the denominator.

\*\*Patients in the placebo arm switched to TransCon PTH following the Week 26 visit.

In June 2023, we announced one-year (Week 52) data from the open-label extension (“OLE”) portion of the Phase 3 PaTHway Trial of TransCon PTH in adults with hypoparathyroidism. PaTHway is a Phase 3 trial of TransCon PTH with a placebo-controlled 26-week blinded portion and a 156-week OLE portion, designed to evaluate the long-term efficacy and safety of TransCon PTH as a potential hormone therapy for adult patients diagnosed with hypoparathyroidism. Of the 82 study participants dosed, 79 completed blinded treatment and entered the OLE, and 78 (59 TransCon PTH/TransCon PTH, 19 placebo/TransCon PTH) completed Week 52. The data showed that treatment with TransCon PTH resulted in sustained improvements through Week 52, as well as safety and tolerability similar to that reported for the initial 26-week blinded portion of the trial. As of June 30, 2024, 74 out of 79 patients continue in the OLE and have exceeded two years of follow-up in the PaTHway Trial.

On January 8, 2023, we announced topline data from PaTHway Japan, a single-arm Phase 3 trial to evaluate the safety, tolerability, and efficacy of TransCon PTH in adults with hypoparathyroidism. The study achieved its primary objective, with topline results consistent with our trials in North America and the EU. Twelve out of thirteen patients met the primary multi-component endpoint, which was defined as serum calcium levels in the normal range (8.3–10.6 mg/dL) and independence from conventional therapy (no active vitamin D and  $\leq 600$  mg/day of calcium). In this trial, TransCon PTH was generally well-tolerated, with no discontinuations related to study drug. As of March 31, 2024, 12 patients continue in the ongoing 3-year extension portion of the PaTHway Japan Trial.

In December 2022, the FDA allowed us to initiate a U.S. expanded access program (“EAP”) for TransCon PTH for eligible adult patients with hypoparathyroidism with prior PTH treatment experience. This EAP program is ongoing.

In September 2022, we announced new Week 110 data from the Phase 2 PaTH Forward Trial showing that long-term therapy with TransCon PTH provided a durable response in adult patients with hypoparathyroidism, as evidenced by maintenance of normal mean serum calcium levels and 93% of patients achieving independence from conventional therapy (no active vitamin D and  $\leq 600$  mg/day of calcium). Fifty-seven subjects exceeded three years of follow-up in the PaTH Forward Trial. As of June 30, 2024, 56 out of the 59 patients originally enrolled in the trial continued in the OLE portion and have exceeded four years of follow-up. Three patients withdrew from the trial for reasons unrelated to safety or efficacy of the study drug.

In March 2022, we announced that top-line data from the randomized, double-blind, placebo-controlled portion of the Phase 3 PaTHway Trial of TransCon PTH in adults with hypoparathyroidism demonstrated statistically significant higher proportion of participants treated with TransCon PTH achieved the primary multi-component endpoint compared to placebo. The primary endpoint, defined as serum calcium levels in the normal range (8.3–10.6 mg/dL) and independence from conventional therapy (no active vitamin D and  $\leq 600$  mg/day of calcium) with no increase in prescribed study drug within the 4 weeks prior to the Week 26 visit, was achieved by 78.7% of TransCon PTH-treated patients (48 of 61), compared to 4.8% for patients (1 of 21) in control group (p-value  $< 0.0001$ ). In addition, all key pre-specified secondary endpoints were met with statistical significance. TransCon PTH was generally well tolerated, with no discontinuations related to study drug. Three patients discontinued during the treatment period, two from the placebo arm and one from the TransCon PTH arm. TransCon PTH-treated patients showed a mean decrease in 24-hour urine calcium excretion into the normal range.

In April 2020, we announced top-line data from the four-week fixed dose, double-blinded portion of PaTH Forward, a global Phase 2 trial evaluating the safety, tolerability and efficacy of TransCon PTH in adult subjects with hypoparathyroidism. A total of 59 subjects were randomized in a blinded manner to receive fixed doses of TransCon PTH at 15, 18 or 21  $\mu\text{g/day}$  or placebo for four weeks using a ready-to-use prefilled pen injector planned for commercial presentation. All doses of TransCon PTH were well-tolerated, and no serious or severe treatment-related adverse events (“TEAEs”), were observed at any point. No treatment-emergent adverse events led to discontinuation of study drug, and the overall incidence of TEAEs was comparable between TransCon PTH and placebo. Additionally, there were no drop-outs during the four-week fixed dose period.

In June 2018, we were granted Orphan Drug Designation (“ODD”) by the FDA, for TransCon PTH for the treatment of hypoparathyroidism. In October 2020, we were granted Orphan designation (“OD”) by the European Commission (“EC”) for TransCon PTH for the treatment of hypoparathyroidism. In July 2021, the Ministry of Health, Labour and Welfare in Japan granted ODD to TransCon PTH for the treatment of hypoparathyroidism.

### ***TransCon CNP***

#### *Market Opportunity in Achondroplasia*

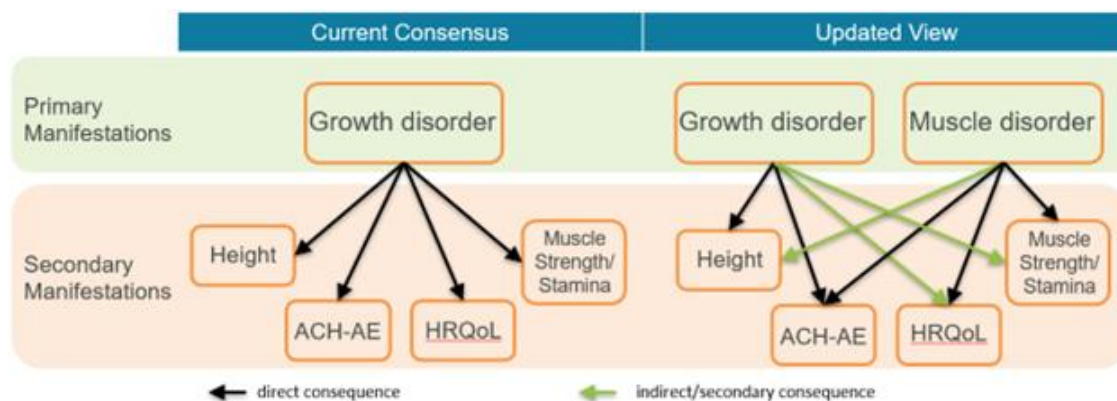
Achondroplasia is the most common genetic form of skeletal dysplasia leading to disproportionate short stature and is associated with a well-delineated range of clinical complications and manifestations, occurring in about one in 10,000 to 30,000 newborns or approximately 250,000 worldwide. Achondroplasia results in severe skeletal complications and comorbidities including spinal stenosis due to premature fusion of the foramen magnum, sleep apnea, chronic ear infections, and muscular complications. Patients often face multiple surgeries to alleviate its many complications. There is significant unmet need for treatments that ameliorate complications and improve quality of life in achondroplasia.

Achondroplasia is primarily caused by gain-of-function variants of the FGFR3 gene resulting in constitutive activation of FGFR3 that leads to an imbalance in the effects of the FGFR3 and C-type natriuretic peptide (“CNP”) signaling pathways. In achondroplasia, mutations in FGFR3 result in constitutive activation, suppressing the proliferation and differentiation of chondrocytes resulting in improper cartilage to bone conversion in the growth plate, and dysfunction in the skeletal muscle. Preclinical and clinical data show that the CNP pathway helps to counteract the effects of the FGFR3 mutation downstream.

In November 2021, BioMarin Pharmaceutical Inc.’s VOXZOGO® (vosoritide) was approved by the FDA and is indicated to increase linear growth in pediatric patients with achondroplasia with open epiphyses. Other companies that are developing therapies for achondroplasia include QED Therapeutics (a BridgeBio company), Sanofi, Ribomic, Tyra Biosciences, and ProLynx.

## Changing the Treatment Paradigm of Achondroplasia

Clinical manifestations of achondroplasia are associated with significant, potentially life-threatening complications and reduced quality of life. While achondroplasia has historically been considered a growth disorder, secondary manifestations beyond linear growth, including reduced muscle strength and stamina, suggest that achondroplasia is also a muscle disorder.



*ACH-AE: Increased incidence of Achondroplasia-related Adverse Events.*

*HRQoL: Reduced Health-Related Quality of Life; Height; Reduced height. Muscle Strength/Stamina; Reduced muscular functionality, including reduced strength and stamina.*

### Our Solution: TransCon CNP

TransCon CNP (navepegritide) is an investigational prodrug of CNP administered once weekly and designed to provide sustained release of active CNP supporting continuous exposure for the treatment of achondroplasia. TransCon CNP is designed to provide effective shielding of CNP from neutral endopeptidase degradation in subcutaneous tissue and the blood compartment, minimize binding of CNP to the NPR-C receptor to decrease clearance, reduce binding of CNP to the NPR-B receptor in the cardiovascular system to avoid hypotension, and release unmodified CNP, which is small enough in size to allow effective penetration into growth plates. Shorter-acting CNP and CNP analogs in development have resulted in high C<sub>max</sub> levels that may cause adverse cardiovascular events. We believe the therapeutically sustained release of TransCon CNP offers advantages that may mitigate this issue, leading to more constant CNP exposure at lower C<sub>max</sub> to correlate with better therapeutic outcomes.

### Clinical Development of TransCon CNP for Achondroplasia

Our ongoing pivotal ApproaCH Trial, ACcomplish Trial, our long-term extension trial AttaCH and COACH, are evaluating the safety and efficacy of TransCon CNP in children with achondroplasia. The ongoing reACHin Trial is evaluating the safety, tolerability, and efficacy of TransCon CNP in infants with achondroplasia (aged 0 to < 2 years at the time of randomization).

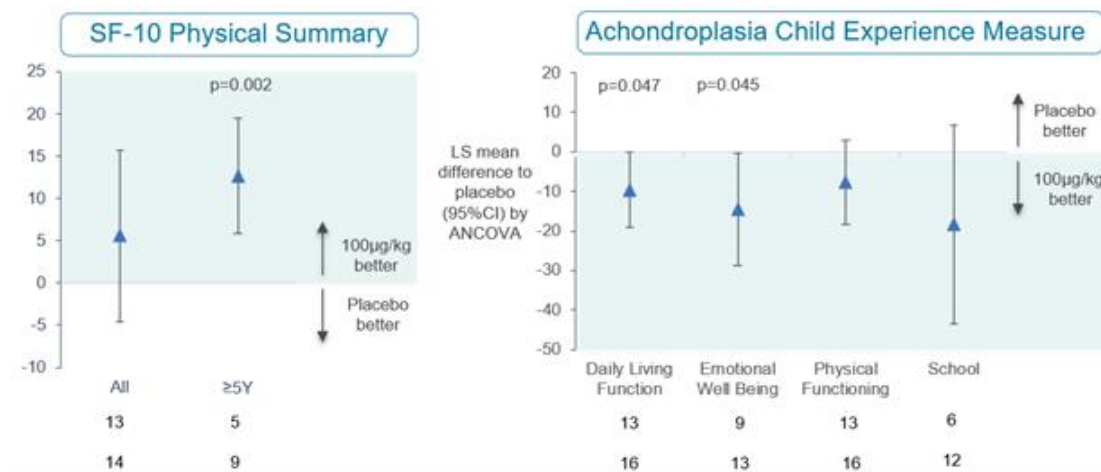
In January 2024, we announced our plan to submit an Investigational New Drug (“IND”) application or similar in the fourth quarter of 2024 to initiate a trial to evaluate TransCon CNP in adults with achondroplasia.

In December 2023, we announced new analyses demonstrating benefits beyond linear growth from the blinded and ongoing OLE portions of ACcomplish, a Phase 2 randomized, double-blind, placebo-controlled, dose-escalation trial of TransCon CNP in children ages 2-10 years with achondroplasia. In the trial, all 57 patients have now completed one year of treatment with TransCon CNP at 100 µg/kg/week, the dose agreed with regulatory agencies for the active arm in our pivotal ApproaCH Trial.

We analyzed available data for patients who only received TransCon CNP at the 100 µg/kg/week dose in either blinded or OLE part and were treated for one year (n=19), compared to those administered placebo for one year (n=15). Results showed that these TransCon CNP-treated patients (data available for 9-16 patients) showed significant improvements in health-related quality of life and disease impacts compared to those receiving placebo (data available for 5-13 patients).

Assessments were performed with the SF-10 (a 10-item non-disease specific survey of a child’s functional health and well-being that is validated in children ages 5 years and older) and the Achondroplasia Child Experience Measure (“ACEM”) a condition-specific clinical outcome measure that assesses the impact of achondroplasia on a child’s health-related quality of life, with statistically significant improved outcome in TransCon CNP-treatment versus placebo for:

- SF-10 Physical Summary (p=0.002, ages 5 years and older)
- ACEM Daily Living Function (p=0.047)
- ACEM Emotional Well-being (p=0.045)



The 46 children switching from placebo or a lower dose of TransCon CNP to the 100 µg/kg/week dose in the OLE demonstrated improved growth after one year of treatment, similar to the growth benefits seen in the 11 children treated with 100 µg/kg/week in the one-year randomized, double-blind period of ACcomplisH.

While the CNP pathway may restore normal growth and skeletal muscle function, delayed initiation of therapy could lead to a permanent height deficit. Clinical use of daily growth hormone injection has consistently demonstrated growth improvements in children with achondroplasia, including catch up growth, however without reports of benefits beyond linear growth. We believe use of TransCon hGH and TransCon CNP together once per week could enable catch up growth beyond normal growth while maintaining benefits on skeletal muscle of continuous CNP exposure. During the fourth quarter of 2023, we filed a Clinical Trial Application for COACH, a Phase 2 open-label single-arm trial evaluating TransCon CNP and TransCon hGH in children with achondroplasia (age 2 to 11 years). The primary objective is to evaluate the treatment effect on linear growth and safety. Secondary objectives are to evaluate treatment effect on quality of life, radiological endpoints, physical functioning, and body composition. The trial plans to enroll approximately 18 patients (treatment naïve, n=12; prior treatment with TransCon CNP (100 µg/kg/week) for at least 1 year, n=6). Week 26 topline data from the COACH Trial are expected in the second quarter of 2025.

During the third quarter of 2023, we filed an IND amendment with the FDA to initiate reACHin, a Phase 2, multicenter, double-blind, randomized, placebo-controlled trial, designed to evaluate the safety, tolerability, and efficacy of 100 µg/kg of TransCon CNP once-weekly for 52 weeks in infants with achondroplasia, aged 0 to < 2 years at the time of randomization.

In September 2023, we announced completion of enrollment in ApproaCH with a total of 84 subjects randomized. U.S. and EU regulatory agencies have endorsed ApproaCH, a global randomized, double-blind, placebo-controlled trial in children ages 2–11 years with achondroplasia, as a pivotal Phase 3 trial. The primary endpoint of the trial is annualized growth velocity at 52 weeks with additional endpoints analyzing achondroplasia-related co-morbidities and quality of life. Topline results from the ApproaCH trial are expected in the third quarter of 2024. In addition, we intend to use the results from ApproaCH in connection with a planned NDA submission to the FDA for TransCon CNP for children with achondroplasia (age 2-11 years) in the first quarter of 2025.

In November 2022, we announced topline results from ACcomplisH, a Phase 2 randomized, double-blind, placebo-controlled, dose-escalation trial evaluating the safety and efficacy of once-weekly TransCon CNP compared to placebo in children with achondroplasia aged two to ten years old.

The ACcomplisH Trial evaluated 57 children with achondroplasia aged 2 to 10 years old, randomized in a 3:1 ratio to receive either sequential ascending doses of once-weekly TransCon CNP (6 µg/kg/week, 20 µg/kg/week, 50 µg/kg/week, 100 µg/kg/week) or placebo for 52 weeks. The trial met its primary objectives, demonstrating that TransCon CNP at 100 µg/kg/week (n=11) was superior to placebo (n=15) on the primary efficacy endpoint of annualized growth velocity (“AGV”) at 52 weeks (p=0.0218). All 57 randomized children completed the blinded portion of ACcomplisH and continued in the OLE portion of ACcomplisH at the 100 µg/kg/week dose. As of June 30, 2024, the first 41 patients completed the OLE portion of the ACcomplisH Trial and transitioned into the Phase 2 AttaCH Trial, a multicenter, long-term, open label extension and 40 continue treatment; 14 patients continue in OLE portion of ACcomplisH.

Additional highlights:

- TransCon CNP demonstrated a consistent dose-dependent increase in AGV across the four dose groups.
- Mean improvements in AGV for TransCon CNP-treated patients were consistent across age groups <5 years and >5 years, with dose response established.
- TransCon CNP at 100 µg/kg/week improved change in achondroplasia-specific height SDS compared to placebo (p=0.0283).
- TransCon CNP was generally well tolerated, with no discontinuations.
- No serious adverse events (“SAEs”) related to treatment were reported; two unrelated SAEs were reported.
- Injections were generally well tolerated with low frequency of injection site reactions (“ISRs”):
  - o 11 mild ISRs (in 8 patients) out of >2,000 injections.
- Investigator-assessed achondroplasia-related AEs were less frequently reported among participants receiving TransCon CNP (31%; 13/42) compared with placebo (60%; 9/15).

In 2019, we initiated the ACHieve Study, a five-year, multi-center natural history study designed to gain insight into the experiences of pediatric patients with achondroplasia. ACHieve is designed to evaluate growth velocity, body proportionality, and comorbidities over time in children with achondroplasia up to eight years old. No study medication was administered in the ACHieve Study. The study ended in the first quarter of 2024.

In February 2019, we were granted ODD by the FDA for TransCon CNP for the treatment of achondroplasia. In July 2020, we received OD from the EC for TransCon CNP for the treatment of achondroplasia.

### **TransCon Product Candidates—Oncology**

#### *Market Opportunity in Oncology*

Cancer continues to be one of the leading causes of mortality. Improved understanding of the cellular and molecular mechanisms involved in anti-tumor immune responses has fueled the rapid growth of immuno-oncology therapeutics. Immune checkpoint inhibitors, such as anti-PD-(L)1 and anti-CTLA-4 antibodies, have provided new therapeutic options for patients.

Despite recent advances, a high need for new treatment options remains for patients who do not respond or respond inadequately to current therapies. In addition to insufficient efficacy, many current treatments are limited by toxicities that result in dose reductions, treatment discontinuations, or long-term health risks to patients.

We believe that one approach to improving efficacy while limiting adverse events is to create long-acting product candidates using our sustained systemic release TransCon technology, allowing for more consistent circulating drug levels and potentially avoiding high peak concentrations that are often associated with toxicity.

Another approach is to target the drug activity into tumors via intratumoral injection using our sustained localized release TransCon hydrogel technology, aiming for high activity in the tumor microenvironment while limiting systemic adverse events. While one intratumoral treatment has been approved for the local treatment of recurrent melanoma, the overall success of intratumoral treatments has been limited to date. This is likely partly due to lack of prolonged intratumoral exposure of active drug levels, resulting in the potential need for more frequent dosing.

### *Our Solution: TransCon Technologies for Oncology*

We believe prolonging the therapeutic activity and targeting the drug activity to the relevant cell types and tissues have the potential to improve treatment outcomes. We believe TransCon is well-suited to improve cancer treatments given the large number of validated targets with known limitations. By applying our unique algorithm for product innovation to clinically validated targets and pathways, we believe TransCon has the potential to improve outcomes currently limited by suboptimal efficacy and systemic toxicity.

We believe TransCon technologies may have the potential to increase the efficacy of small molecules, peptides and proteins without increasing toxicity, which could offer the potential to treat more patients with new combination and multi-agent regimens that would not otherwise be feasible.

We are currently investigating two clinical-stage product candidates designed to activate the patient's own immune system to eradicate malignant cells. We believe our approach, if successfully developed, has the potential to improve the efficacy of systemically administered, clinically validated therapies while limiting adverse effects.

Similarly, with the potential to achieve sustained local release at predictable levels, we believe TransCon hydrogel product candidates may allow for improved efficacy and reduced dosing frequency of intratumorally administered therapies, potentially enabling treatments of multiple tumor types, including those that cannot be easily accessed for frequent injection.

### *Development of TransCon Product Candidates in Oncology*

Our TransCon product candidates in oncology are designed to provide sustained systemic or intratumoral administration, which we believe could provide potent and durable anti-tumor efficacy. Our nonclinical studies have shown sustained activation of cytotoxic immune cells that resulted in robust anti-tumor responses by TransCon product candidates using infrequent administration.

Two of our oncology product candidates, TransCon TLR7/8 Agonist and TransCon IL-2 b/g, are now in clinical development. In addition, we believe that a combination of TransCon TLR7/8 Agonist and TransCon IL-2 b/g may have the potential to produce greater anti-tumor activity than either candidate alone.

### ***TransCon TLR7/8 Agonist for sustained localized release***

TransCon TLR7/8 Agonist is an investigational long-acting prodrug, designed for sustained intratumoral release of resiquimod, a small molecule agonist of TLR 7 and 8. It is designed to provide sustained and potent activation of the innate immune system in the tumor and tumor draining lymph node for weeks following a single intratumoral injection and to have a low risk of systemic toxicity. The transcendIT-101 Trial, a Phase 1/2 clinical trial to evaluate the safety and efficacy of TransCon TLR7/8 Agonist in locally advanced or metastatic solid tumors, alone or in combination with pembrolizumab, has completed dose escalation and is enrolling patients in four indication-specific cohorts where increased TLR7/8 activity has potential to improve innate and adaptive immune activation and host defense against cancers: head and neck squamous cell carcinoma (HNSCC), HPV-associated cancers, melanoma, and cutaneous squamous cell carcinoma (cSCC). Initial data from these cohorts are expected by the end of 2024.

In May 2023, we announced additional follow-up from the transcendIT-101 Trial indicating further clinical activity in patients receiving TransCon TLR7/8 Agonist as monotherapy or in combination with pembrolizumab. Enrollment continues in the Phase 2 portion of transcendIT-101 at the recommended Phase 2 dose ("RP2D"). Updated clinical data indicate intratumoral TransCon TLR7/8 Agonist was generally well tolerated as monotherapy or when used in combination with pembrolizumab. In addition, monotherapy confirmed response in injected and non-injected tumors was observed, indicating abscopal effect and ability to induce systemic immune effects.

In November 2022, we announced new data (cutoff date of September 21, 2022) from the dose-escalation portion of transcendIT-101. All 23 of the patients enrolled in the dose escalation portion of the trial had advanced or metastatic solid tumors that had progressed on prior treatments, 9 in the monotherapy cohort (intratumoral TransCon TLR7/8 Agonist alone) and 14 in the combination therapy cohort (intratumoral TransCon TLR7/8 Agonist plus the checkpoint inhibitor pembrolizumab). Two dose levels were evaluated: 0.3 mg/lesion and 0.5 mg/lesion. The RP2D was declared at 0.5 mg/lesion for up to two lesions, which is being evaluated in four indication specific cohorts.



### ***TransCon IL-2 b/g for sustained systemic release***

TransCon IL-2 b/g (onvapegleukin alfa) is an investigational long-acting prodrug designed to improve cancer immunotherapy through sustained release of an IL-2 variant that selectively activates IL-2 b/g, with minimal binding to IL-2R $\alpha$ . The IL-Believe Trial, a Phase 1/2 clinical trial to evaluate the safety and efficacy of TransCon IL-2 b/g in locally advanced or metastatic solid tumors, alone or in combination with pembrolizumab or standard of care chemotherapy, has completed dose escalation and is enrolling patients in multiple indication-specific dose expansion cohorts, including platinum-resistant ovarian cancer (PROC), cervical cancer, melanoma, non-small cell lung cancer (NSCLC), and small cell lung cancer (SCLC) at the RP2D. Initial data from these cohorts are expected by the end of 2024.

In June 2024, we reported interim results from our ongoing Phase 1/2 IL-Believe Trial of TransCon IL-2 b/g. Data included the first presentation of Phase 2 dose expansion Cohort 4 (TransCon IL-2  $\beta\gamma$  in combination with TransCon TLR7/8 Agonist) in post anti-PD-1 melanoma and new analyses of patients from dose escalation cohorts with prior disease progression on checkpoint inhibitors, along with biomarker studies correlating cytotoxic immune cell expansion and clinical benefit. As of the April 16, 2024, data cutoff, confirmed clinical responses were observed in 40% (two out of five) of efficacy-evaluable patients from Cohort 4, suggesting potential synergy of our two novel immunotherapy agents in patients who did not derive sufficient benefit from checkpoint inhibitors. Of efficacy-evaluable patients with prior disease progression on checkpoint inhibitors to date in the IL-Believe Trial, confirmed clinical responses (per RECIST v1.1) were observed in 45% (five out of eleven) administered TransCon IL-2  $\beta\gamma$  doses  $\geq 80$   $\mu\text{g}/\text{kg}$  every 3 weeks, suggesting clinical benefit in treatment-resistant settings (monotherapy (n=4): 1 confirmed partial response (“PR”) in colorectal cancer; combination with pembrolizumab (n=2): 1 confirmed complete response and 1 confirmed PR in small-cell lung cancer; combination with TransCon TLR7/8 Agonist (n=5): 2 confirmed PRs in melanoma). In this trial, TransCon IL-2  $\beta\gamma$  alone or in combination with pembrolizumab or TransCon TLR7/8 Agonist was generally well tolerated with no new safety signals.

During the fourth quarter of 2023, the first patient was dosed with the combination of TransCon IL-2 b/g and TransCon TLR7/8 Agonist in the post PD-1 melanoma dose expansion cohort in the IL-Believe Trial.

In October 2023, we announced new and updated data from the ongoing IL-Believe Trial. Forty-six patients were enrolled into dose escalation cohorts: 25 to monotherapy and 21 to combination therapy. As of the August 15, 2023, data cutoff, anti-tumor clinical responses were observed with TransCon IL-2 b/g monotherapy (colorectal cancer with PR) or in combination with pembrolizumab (small cell lung cancer, one with confirmed PR and one ongoing with unconfirmed complete response) in heavily pre-treated patients who previously progressed on checkpoint inhibitors. TransCon IL-2 b/g every three weeks was generally well-tolerated, with no meaningful effect on Tregs and eosinophils.

In September 2023, we announced completion of Phase 1 dose escalation in combination with pembrolizumab of the IL-Believe Trial with a total of 21 patients enrolled and RP2D determined at 120  $\mu\text{g}/\text{kg}$  IV every three weeks. Twenty-one patients were enrolled.

In May 2023, we announced completion of the Phase 1 monotherapy dose escalation of the IL-Believe Trial with RP2D determined at 120  $\mu\text{g}/\text{kg}$  IV every three weeks with 25 heavily pre-treated patients enrolled and a median of four prior lines of systemic therapies.

### ***Other Development Plans***

To further evaluate safety and anti-tumor efficacy of TransCon TLR7/8 Agonist and TransCon IL-2 b/g, we are also evaluating these product candidates as neoadjuvant therapy in the ongoing randomized Phase 2 BelieveIT-201 trial in resectable locally advanced head and neck squamous cell carcinoma.

### **Strategic Collaborations**

We also engage in strategic collaborations to further leverage our TransCon technologies in certain geographies with market-leading biopharmaceutical companies. These collaborations aim to make promising treatment options available to more patients and to further monetize both our TransCon technologies and our internal product candidates, particularly into therapeutic areas where we believe a partner may have more expertise, capability, and capital. In addition, we may choose to pursue a collaboration to develop and market our internal, wholly owned product candidates in geographic markets outside our core focus areas of the United States and Europe.

In November 2023, we announced that we entered into an exclusive license agreement with Teijin Limited for the further development and commercialization of TransCon hGH, TransCon PTH, and TransCon CNP for endocrinology rare disease in Japan. Under the terms of the agreement with Teijin Limited, we received an upfront payment of \$70 million, with additional development and regulatory milestones of up to \$175 million, transfer pricing and commercial milestones. In addition, we are eligible to receive royalties on net sales in Japan, of up to mid-20's percent, varying by product.

## Strategic Investments

### *VISEN Pharmaceuticals*

In November 2018, we announced the formation of VISEN, a company established to develop and commercialize our endocrinology rare disease therapies in the People's Republic of China, Hong Kong, Macau, and Taiwan ("Greater China"). In connection with the formation of VISEN, we granted VISEN exclusive rights to develop and commercialize certain product candidates based on our proprietary TransCon technologies, including TransCon hGH, TransCon PTH and TransCon CNP, in Greater China for use in all human indications, subject to certain exceptions. As consideration for the rights granted to VISEN, we received 50% ownership in the outstanding shares of VISEN and concurrently with the rights we granted to VISEN, entities affiliated with Vivo Capital and Sofinnova Ventures purchased shares in VISEN for an aggregate purchase price of \$40 million in cash. In January 2021, we invested additional \$12.5 million in VISEN as part of VISEN's \$150 million Series B financing. Following the Series B financing, we retained 43.93% of VISEN's issued and outstanding shares.

In August 2024, VISEN announced topline data from the 26-week randomized, double-blind, placebo-controlled portion of the Phase 3 PaTHway China Trial of Palopegteriparatide (TransCon PTH) in adults with chronic hypoparathyroidism. A statistically significantly higher proportion of patients treated with palopegteriparatide achieved the primary multi-component endpoint compared to placebo (p-value <0.0001). Palopegteriparatide was generally safe and well-tolerated, with no discontinuations related to study drug. Results were consistent with those announced by Ascendis Pharma for its palopegteriparatide Phase 3 trial.

In March 2024, VISEN announced that the BLA for lonapegsomatropin (TransCon hGH) was accepted by the China National Medical Products Administration. Lonapegsomatropin is the first once-weekly administered growth hormone approved by both the FDA and EMA for the treatment of pediatric GHD.

In November 2023, VISEN announced topline results from the Phase 2 ACcomplish China Trial in children with achondroplasia aged 2 to 10 years. The trial met its primary objectives, demonstrating that TransCon CNP at 100 µg/kg/week was superior to placebo on the primary efficacy endpoint of AGV at 52 weeks (p=0.018).

In November 2022, VISEN announced data from its pivotal Phase 3 study of TransCon hGH in children with GHD in China. The trial achieved its primary endpoint; patients treated with TransCon hGH demonstrated greater annualized height velocity at 52-weeks (p=0.0010) compared to patients treated with daily growth hormone with comparable safety and tolerability to daily growth hormone.

In June 2022, VISEN announced it had completed enrollment of the Phase 3 PaTHway China Trial of TransCon PTH.

### *Market Opportunity in China*

China is the second largest pharmaceutical market in the world after the United States and represents one of the fastest growing pharmaceutical markets worldwide. In recent years, the Chinese government has initiated a number of regulatory reforms that are expected to accelerate drug development, as well as drive growth and demand for new therapeutics in China. In addition to joining an international organization that standardizes regulations for clinical development, the National Medical Products Administration has introduced initiatives such as fast track review for drugs for unmet medical needs and adopted new rules that streamline the drug approval process in China for global companies.

The purpose of our investment in VISEN is to support our strategy to extend our endocrinology rare disease portfolio globally and establish a presence in China in partnership with collaborators who have significant experience and knowledge of the biopharmaceutical opportunity in China.

### *Rights Agreements*

Under three exclusive license agreements, each effective November 7, 2018, and as amended January 4, 2021, between the Company and VISEN (collectively, the "Rights Agreements"), VISEN must use diligent efforts to develop and commercialize licensed products in Greater China. Additionally, we and VISEN will conduct certain research and development activities allocated to the respective party under a research and technical development plan, and VISEN will reimburse us for costs of conducting such activities, including costs of our personnel committed to performing such activities in Greater China.

We entered into a clinical supply agreement with VISEN in 2018 to provide product supply for use in conducting clinical trials in Greater China. Additionally, during 2023, we entered into a commercial supply agreement governing commercial supply of licensed product (TransCon hGH) to VISEN on the terms and conditions set forth in the Rights Agreements.

Under the Rights Agreements, we agreed not to research, develop, or commercialize competing products in Greater China, and VISEN agreed not to grant certain rights under its interest in any inventions or intellectual property arising out of the activities conducted under the Rights Agreements to third-parties, in each case, under the terms and conditions specified in the Rights Agreements. We will have the right to exploit inventions and intellectual property arising out of the activities conducted under the Rights Agreements outside of Greater China. Additionally, we granted VISEN a right of first negotiation to develop and commercialize certain of our endocrinology products in Greater China.

The Rights Agreements continue in effect for as long as a valid claim of a licensed patent exists in Greater China. VISEN may terminate a Rights Agreement for convenience, for uncured material breach by us of a Rights Agreement and for our bankruptcy or insolvency-related events. We may terminate a Rights Agreement for certain specified material breaches thereof by VISEN, in the event VISEN undergoes a change of control in favor of a competitor, if VISEN challenges the validity of any of the licensed patents and for VISEN's bankruptcy or insolvency-related events.

#### *Amended and Restated Shareholders Agreement*

In connection with the Company's investment in VISEN, on January 8, 2021, the Company entered into an Amended and Restated Shareholders Agreement (the "Amended Shareholders Agreement"), amending and restating the Shareholders Agreement dated November 7, 2018, between the Company and the parties set forth therein (the "Shareholders Agreement"). In addition to rights previously granted under the Shareholders Agreement, under the Amended Shareholders Agreement, the Company has the right to designate two individuals for election to the board of directors of VISEN, which individuals are Jan Møller Mikkelsen and Michael Wolff Jensen. In addition, VISEN has agreed that certain specified events (including certain liquidation events) shall require the approval of (i) shareholders of VISEN holding at least 50% of VISEN's Series B preferred shares, (ii) shareholders of VISEN holding at least 60% of VISEN's Series A preferred shares and/or (iii) certain members of VISEN's board of directors. The Amended Shareholders Agreement can be terminated by written agreement among the holders of at least 60% of VISEN's Series A preferred shares and at least 50% of VISEN's Series B preferred shares.

#### *Eyconis*

In January 2024, we announced the formation and launch with Frazier Life Sciences of Eyconis, Inc., a separate company created to develop, manufacture, and commercialize TransCon ophthalmology assets globally, together with a \$150 million commitment from an investor syndicate that includes Frazier, RA Capital Management, venBio, and HealthQuest Capital.

We have granted Eyconis exclusive rights to develop and commercialize TransCon ophthalmology products globally and received an equity position in the newly formed company. In addition, we will be eligible to receive development, regulatory, and sales milestone payments, plus single digit royalties on global net sales of commercialized products, if any. Eyconis will initially be based in Redwood City, California, and certain employees of Ascendis have joined the newly formed company.

## Results of Operations

### Financial Highlights (unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
	(EUR'000)			
Revenue	35,998	47,393	131,892	80,982
Gross profit	24,533	34,464	112,858	63,431
Operating expenses <sup>(1)</sup>	157,790	175,302	295,260	347,954
Operating profit/(loss)	(133,257)	(140,838)	(182,402)	(284,523)
Net profit/(loss) for the period	(109,380)	(121,433)	(240,415)	(232,346)
Cash flows from/(used in) operating activities	(61,307)	(153,128)	(162,890)	(300,172)

(1) Operating expenses comprise research and development costs and selling, general and administrative expenses.

Compared to the three and six months ended June 30, 2023, revenue for the three and six months ended June 30, 2024, primarily benefited from continued demand growth for SKYTROFA<sup>®</sup> (lonapegsomatropin-tcgd) in the U.S., non-cash license revenue related to our exclusive license agreement with Eyconis in January 2024, and launch of YORVIPATH<sup>®</sup> (palopegteriparatide) in Europe, partly offset by a negative adjustment to prior periods estimates and assumptions for sales deductions related to sale of SKYTROFA.

Operating loss for the three and six months ended June 30, 2024, represented a decrease of €7.6 million and €102.1 million, compared to the same period last year, primarily attributable to lower operating expenses. In addition, the six months ended June 30, 2024 was positively impacted by higher revenue from commercial sales and from non-cash license revenue of €25.6 million related to our exclusive license agreement with Eyconis in January 2024.

We had a net loss of €240.4 million for the six months ended June 30, 2024, compared to a net loss of €232.3 million for the same period last year. Total equity presented a negative balance of €321.1 million as of June 30, 2024, compared to a negative balance of €145.7 million as of December 31, 2023.

Further details about our results of operations and cash flows are described in the following sections.

### Comparison of the Three and Six Months Ended June 30, 2024 and 2023 (unaudited)

#### Revenue

Revenue for the three and six months ended June 30, 2024, were €36.0 million and €131.9 million, representing a decrease of €11.4 million and an increase of €50.9 million, compared to the same period last year.

The €50.9 million increase for the six months ended June 30, 2024 was primarily attributable to sales of SKYTROFA, recognition of non-cash license revenue of €25.6 million related to our exclusive license agreement with Eyconis in January 2024, and the launch of YORVIPATH, which we began shipping to customers in Europe in the first quarter of 2024. Revenue for the six months ended June 30, 2024, was negatively impacted by an adjustment to estimates and assumptions for sales deductions of €7.6 million, which were attributable to periods prior to January 1, 2024. The adjustment was primarily attributable to a different payer and rebate mix than anticipated, and which provisions for prior periods were based upon.

The €11.4 million decrease for the three months ended June 30, 2024, was primarily driven by a negative adjustment to prior periods estimates and assumptions for sales deductions of €27.1 million, where €19.5 million and €7.6 million were attributable to the three months ended March 31, 2024 and periods prior to January 1, 2024, respectively, partly offset by increased sales of SKYTROFA and the launch of YORVIPATH. In addition, the three months ended June 30, 2024 was negatively impacted by lower revenue of €6.9 million, associated with delivering of services under our license and collaboration agreements.

The development in quarterly revenue from sale of commercial products was as follows:

	Three Months Ended,				
	June 30, 2023	September 30, 2023	December 31, 2023	March 31, 2024	June 30, 2024
	(EUR'000)				
Sale of commercial products	35,895	46,968	64,249	66,499	31,389

## Cost of Sales

Cost of sales for the three and six months ended June 30, 2024, was €11.5 million and €19.0 million, representing a decrease of €1.5 million and an increase €1.5 million, compared to the same period last year. This development was primarily attributable to higher volume of commercial sales for the three and six months ended June 30, 2024, offset by lower costs associated with delivering of services under our license and collaboration agreements compared to the same period last year.

## Research and Development Costs

The following table specifies external project costs on the development pipeline and other research and development costs.

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
	(EUR'000)		(EUR'000)	
<b>External project costs</b>				
TransCon hGH	12,491	12,882	24,308	30,888
TransCon PTH	5,876	10,100	(442)	22,614
TransCon CNP	15,515	13,473	31,259	24,418
TransCon IL-2 B/γ	7,973	8,532	14,937	19,728
TransCon TLR7/8 Agonist	2,290	12,081	5,199	20,574
Ophthalmology	—	3,038	—	4,759
Other project costs <sup>(1)</sup>	736	36	1,959	544
<b>Total external project costs</b>	<b>44,881</b>	<b>60,142</b>	<b>77,220</b>	<b>123,525</b>
<b>Other research and development costs</b>				
Employee costs	33,513	36,002	64,779	68,583
Other costs	3,675	5,758	8,518	13,203
Depreciation	1,409	3,119	3,648	5,823
<b>Total other research and development costs</b>	<b>38,597</b>	<b>44,879</b>	<b>76,945</b>	<b>87,609</b>
<b>Total research and development costs</b>	<b>83,478</b>	<b>105,021</b>	<b>154,165</b>	<b>211,134</b>

(1) For the three and six months ended June 30, 2023, other project costs of €3.8 million and €6.6 million were reclassified to Ophthalmology and TransCon IL-2 b/g, with €3.0 million and €4.8 million respectively and €0.8 million and €1.8 million, respectively.

R&D costs for the three and six months ended June 30, 2024, were €83.5 million and €154.2 million, representing a decrease of €21.5 million and €57.0 million, compared to the same period last year. This decrease was primarily due to:

- Maturity of our endocrinology rare disease pipeline, especially regarding TransCon hGH and TransCon PTH. In addition, the six months ended June 30, 2024 includes reversal (income) of prior period write-downs of pre-launch inventories for TransCon PTH of €10.6 million due to the launch of YORVIPATH in the EU in the first quarter of 2024;
- Lower product development activities on our TransCon TLR7/8 Agonist and TransCon IL-2 b/g programs, partly offset by increased clinical trial activities;
- Ceasation of ophthalmology expenses, including employee costs, due to the grant of exclusive rights to develop and commercialize TransCon ophthalmology to Eyconis in January 2024; partly offset by higher costs for TransCon CNP, including write-downs on pre-launch inventories.

The development in quarterly external project costs was as follows:

	Three Months Ended,				
	June 30, 2023	September 30, 2023	December 31, 2023	March 31, 2024	June 30, 2024
	(EUR'000)				
<b>External project costs</b>					
TransCon hGH	12,882	20,892	12,197	11,816	12,491
TransCon PTH	10,100	10,600	7,366	(6,319)	5,876
TransCon CNP	13,473	18,627	15,570	15,744	15,515
TransCon IL-2 b/g	8,532	10,279	1,987	6,964	7,973
TransCon TLR7/8 Agonist	12,081	7,843	5,066	2,910	2,290
Ophthalmology	3,038	5,543	6,419	—	—
Other project costs	36	129	353	1,224	736
<b>Total external project costs</b>	<b>60,142</b>	<b>73,913</b>	<b>48,958</b>	<b>32,339</b>	<b>44,881</b>

### *Selling, General and Administrative Expenses*

The following table specifies selling, general and administrative expenses:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
	(EUR'000)		(EUR'000)	
<b>Selling, general and administrative expenses</b>				
Employee costs	35,409	30,225	68,951	57,698
Other costs	36,886	38,876	68,683	76,449
Depreciation	2,017	1,180	3,462	2,673
<b>Total selling, general and administrative expenses</b>	<b>74,312</b>	<b>70,281</b>	<b>141,096</b>	<b>136,820</b>

Selling, general and administrative (“SG&A”) expenses for the three and six months ended June 30, 2024, were €74.3 million and €141.1 million, representing an increase of €4.0 million and €4.3 million, compared to the same period last year. This increase was primarily due to higher employee costs, including the impact from commercial expansion. Other costs decreased primarily due to lower pre-launch and other administrative costs, partly offset by higher costs related to the European launch activities.

The development in quarterly SG&A expenses was as follows:

	June 30, 2023	Three Months Ended,			
		September 30, 2023	December 31, 2023	March 31, 2024	June 30, 2024
		(EUR'000)			
<b>Selling, general and administrative expenses</b>					
Employee costs	30,225	28,381	29,627	33,543	35,409
Other costs	38,876	34,132	32,016	31,795	36,886
Depreciation	1,180	1,101	2,332	1,445	2,017
<b>Total selling, general and administrative expenses</b>	<b>70,281</b>	<b>63,614</b>	<b>63,975</b>	<b>66,783</b>	<b>74,312</b>

### Finance Income and Finance Expenses

The following table specifies the result of net finance income and expenses, further disaggregated into cash and non-cash items:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
	(EUR'000)			
<b>Net finance income/(expenses)</b>				
Finance income	49,052	35,761	14,395	80,374
Finance expenses	(19,624)	(9,334)	(58,553)	(18,652)
<b>Total net finance income/(expenses)</b>	<b>29,428</b>	<b>26,427</b>	<b>(44,158)</b>	<b>61,722</b>
<b>Specified in cash and non-cash items</b>				
<u>Cash items</u>				
Finance income received	2,677	4,524	6,265	8,403
Finance expenses paid	(6,837)	(6,841)	(7,714)	(7,746)
<u>Non-cash items</u>				
Remeasurement gain/(loss) of financial liabilities	46,375	30,384	(7,620)	71,565
Currency gain/(loss)	(4,791)	1,085	(13,411)	563
Amortization charges, accruals and other items	(7,996)	(2,725)	(21,678)	(11,063)
<b>Total net finance income/(expenses)</b>	<b>29,428</b>	<b>26,427</b>	<b>(44,158)</b>	<b>61,722</b>

The development in non-cash items was primarily due to remeasurement of financial liabilities, which influenced especially the six months ended June 30, 2024. The increase in amortization charges, accruals and other items primarily relates to amortization charges and interests on royalty funding liabilities, which we entered into in September 2023. In addition, the development in currency gain/(loss) was primarily driven by conversion of U.S. dollar denominated monetary positions into Euro, primarily cash and cash equivalents, convertible notes and royalty funding liabilities.

## Liquidity and Capital Resources

Our liquidity and capital resources comprise cash and cash equivalents, which as of June 30, 2024, amounted to €258.7 million.

Our expenditures primarily relate to continued development of our endocrinology rare disease and oncology therapeutic areas, the commercialization of SKYTROFA and YORVIPATH, and expenses made in anticipation of potential future product launches. We manage our liquidity risk by maintaining adequate cash reserves and banking facilities in line with our cash-forecasts. We monitor the risk of a shortage of funds through liquidity planning tools, to ensure sufficient funds are available to settle liabilities as they become due.

As of June 30, 2024, the unaudited condensed consolidated interim statements of financial position presented a negative balance of equity of €321.1 million. Under Danish corporate law, as Ascendis Pharma A/S, the parent company of the Company holds a positive balance of equity, the Company is currently not subject to legal or regulatory requirements to re-establish the balance of equity. There is no direct impact from the negative balance of equity to the liquidity and capital resources.

Based on our current operating plan, we believe that our existing capital resources as of June 30, 2024, will be sufficient to meet our projected cash requirements for at least twelve months from the date of this report. However, our operating plan may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned.

Historically, we have funded our operations primarily through issuance of preference shares, ordinary shares (including public offerings and exercise of warrants), convertible debt securities, payments to us made under collaboration agreements, and our royalty funding agreement. Including our initial public offering, since February 2015, we have completed public offerings of American Depositary Shares (“ADSs”) with net proceeds of \$2,256.6 million (or €1,968.4 million at the time of the offerings).

In March 2022, we issued an aggregate principal amount of \$575.0 million of fixed rate 2.25% convertible notes. The coupon interest is payable semi-annually. Unless earlier converted or redeemed, the convertible notes will mature on April 1, 2028. We used \$116.7 million (€105.3 million) of the net proceeds from the offering in March 2022 to repurchase 1,000,000 ADSs representing our ordinary shares. The holding of treasury shares is disclosed in Note 9, “Treasury Shares.”

In September 2023, we entered into a \$150.0 million capped synthetic royalty funding agreement (the “Royalty Pharma Agreement”) with Royalty Pharma (the “Purchaser”). Under the terms of the Royalty Pharma Agreement, in exchange for the Purchaser’s payment of a cash purchase price of \$150.0 million at closing (the “Purchase Price”), we have agreed to sell to the Purchaser its right to receive payment in full of 9.15% on net U.S. SKYTROFA revenue, beginning on January 1, 2025 (the “Revenue Interest Payments”). The Revenue Interest Payments to the Purchaser will cease upon reaching a multiple of the Purchase Price of 1.925x, or 1.65x if the Purchaser receives Revenue Interest Payments in that amount by December 31, 2031. The Royalty Pharma Agreement includes a buy-out option under various terms and conditions.

In September 2024, we entered into a \$150.0 million capped synthetic royalty funding agreement (the “Yorvipath Agreement”) with the Purchaser. Under the terms of the Yorvipath Agreement, in exchange for the Purchaser’s payment of a cash purchase price of \$150.0 million at closing (the “Purchase Amount”), we have agreed to sell to the Purchaser its right to receive payment in full of 3% on net U.S. YORVIPATH revenue (the “Revenue Payments”). The Revenue Payments to the Purchaser will cease upon reaching a multiple of the Purchase Amount of 2.0x, or 1.65x if the Purchaser receives Revenue Payments in that amount by December 31, 2029. The Yorvipath Agreement includes a buy-out option under various terms and conditions.

Further details regarding our borrowings are provided in Note 10, “Financial Assets and Liabilities.”

For additional description of our cash requirements, public offerings, expense structure and commitments, refer to “Item 5B. Liquidity and Capital Resources,” set forth in our Annual Report on Form 20-F filed with the Securities and Exchange Commission on February 7, 2024.

Our future funding requirements will depend on many factors, including, but not limited to:

- the extent to which we are able to generate product revenue from sales of our products;
- the manufacturing, selling and marketing costs associated with our products and product candidates, if approved, including the cost and timing of building our sales and marketing capabilities;
- the timing, receipt, and amount of sales of, or royalties on, TransCon hGH, TransCon PTH, and any future products;



- the sales price and the availability of adequate third-party coverage and reimbursement for our products and product candidates, if approved;
- the costs related to manufacturing of our products and product candidates, including the timing of when we incur such costs;
- our ability to establish and maintain strategic partnerships, licensing or other arrangements and the financial terms of such agreements;
- our ability to collect payments which are due to us from customers and collaboration partners (if any), which in turn is impacted by the financial standing of any such customers and collaboration partners;
- the progress, timing, scope, results and costs of our preclinical studies and clinical trials and manufacturing activities for our product candidates that have not been licensed, including the ability to enroll patients in a timely manner for clinical trials;
- the time and cost necessary to obtain regulatory approvals for our product candidates and the costs of post-marketing studies that could be required by regulatory authorities;
- the cash requirements of any future acquisitions or discovery of product candidates;
- the number and scope of preclinical and discovery programs that we decide to pursue or initiate;
- the potential acquisition and in-licensing of other technologies, products or assets;
- the time and cost necessary to respond to technological and market developments, including further development of our TransCon technologies;
- the achievement of development, regulatory and commercial milestones resulting in the payment to us from collaboration partners of contractual milestone payments and the timing of receipt of such payments, if any;
- our progress in the successful commercialization and co-promotion of our products and product candidates, if approved, and our efforts to develop and commercialize our other existing product candidates;
- the market opportunities and patient populations for our products and product candidates, if approved, including with respect to TransCon PTH, and our ability to obtain market acceptance of our products and product candidates, if approved;
- the costs of filing, prosecuting, maintaining, defending and enforcing any patent claims and other intellectual property rights, including litigation costs and the outcome of such litigation, including costs of defending any claims of infringement brought by others in connection with the development, manufacture or commercialization of our product candidates; and
- the extent to which we purchase ADSs prior to settlement for such shares under our equity incentive plans.

Additional funds may not be available if we need them or on terms that are acceptable to us, or at all. If adequate funds are not available to us on a timely basis, we may be required to delay, limit, scale back or cease our research and development and commercialization activities, preclinical studies and clinical trials.

The following table summarizes our cash flows for the six months ended June 30, 2024 and 2023:

	2024	Six Months Ended June 30, 2023 (EUR'000)	Change
<b>Cash flows from/(used in)</b>			
Operating activities	(162,890)	(300,172)	137,282
Investing activities	7,763	257,603	(249,840)
Financing activities	15,968	(3,011)	18,979
<b>Net increase/(decrease) in cash and cash equivalents</b>	<b>(139,159)</b>	<b>(45,580)</b>	<b>(93,579)</b>

### ***Cash Flows from / (used in) Operating Activities***

Cash flows used in operating activities for the six months ended June 30, 2024, were €162.9 million, representing a decrease of €137.3 million compared to the same period last year. This improvement is primarily related to higher commercial revenue and reduced operating expenditures, and to further adjustment for changes to non-operating financial income and expenses, taxes and non-cash items of total €82.6 million. In addition, the change in cash flows from operations was positively impacted by working capital balances with €54.6 million.

### ***Cash Flows from / (used in) Investing Activities***

Cash flows from investing activities for the six months ended June 30, 2024, were €7.8 million, representing a decrease of €249.8 million compared to the same period last year. This decrease was primarily attributable to €251.8 million higher net settlements of marketable securities in 2023 in line with our liquidity management strategy.

### ***Cash Flows from / (used in) Financing Activities***

Cash flows from financing activities for the six months ended June 30, 2024 were €16.0 million, representing an increase of €19.0 million compared to the same period last year. This increase was primarily attributable to increased warrant exercise activity of €19.5 million.

### **Off-balance Sheet Arrangements**

We have not entered into any off-balance sheet arrangements or any holdings in variable interest entities.

### **Qualitative Disclosures about Market Risk**

Our activities expose us to financial risks of changes in foreign currency exchange rates, inflation rates and interest rates. We do not enter into derivative financial instruments to manage our exposure to such risks. Further, we are exposed to credit risk, equity risk and liquidity risk. For a description of our exposure to liquidity risks, including risks associated with the royalty funding liabilities and processes for managing these risks, please refer to “Liquidity and Capital Resources,” set forth above, and maturity analysis for non-derivative financial liabilities provided in Note 10, “Financial Assets and Liabilities.”

### ***Foreign Currency Risk***

We are exposed to foreign exchange risk arising from various currency exposures, primarily with respect to the U.S. Dollar. While we generate revenue in Euro, a significant portion of our revenue is denominated in U.S. Dollars. Similarly, a significant portion of our operating expenses are denominated in U.S. Dollars. In addition, our outstanding convertible notes and royalty funding liabilities are denominated in U.S. Dollar. We seek to minimize our exchange rate risk by maintaining cash positions in the currencies in which we expect to incur the majority of our future expenses and we make payments from those positions.

### ***Interest Rate Risk***

Outstanding convertible notes comprise a 2.25% coupon fixed rate structure. In addition, the interest rate on lease liabilities is fixed at the lease commencement date. Future indebtedness, including those related to lease arrangements, if any, may be subject to higher interest rates. In addition, future interest income from interest-bearing bank deposits may fall short of expectations due to changes in interest rates.

Derivative liabilities are measured at fair value through profit or loss. Accordingly, since the fair value is exposed from the development in interest rates, the profit or loss is exposed to volatility from such development.

### ***Inflation Risk***

Inflation affects us as our vendors may pass on any increased costs to us and accordingly increase our R&D costs, SG&A expenses and cost of manufacturing. We do not believe that inflation had a material impact on our results of operation for the three and six months ended June 30, 2024.

### ***Credit Risk***

We have adopted an investment policy with the primary purpose of preserving capital, fulfilling our liquidity needs and diversifying the risks associated with cash, cash equivalents and marketable securities. Our investment policy establishes minimum ratings for institutions with which we hold cash, cash equivalents and marketable securities, as well as rating and concentration limits for marketable securities held. All material counterparties are considered creditworthy. While the concentration of credit risk may be significant, the credit risk for each individual counterparty is considered to be low. Our exposure to credit risk primarily relates to cash and cash equivalents. The credit risk on our bank deposits is limited because the counterparties holding significant deposits are banks with high credit-ratings (minimum A3/A-) assigned by international credit-rating agencies.

We maintain the majority of our cash and cash equivalents in accounts with major financial institutions, and our deposits at these institutions exceed insured limits. Market conditions can impact the viability of these institutions. In the event of failure of any of the financial institutions where we maintain our cash and cash equivalents, there can be no assurance that we would be able to access uninsured funds in a timely manner or at all. Any inability to access or delay in accessing these funds could adversely affect our business and financial position. The banks are reviewed on a regular basis and deposits may be transferred during the year to mitigate credit risk.

In order to mitigate the concentration of credit risks on bank deposits and to preserve capital, a portion of the bank deposits may be placed into marketable securities. Our investment policy, approved by the Board, only allows investment in marketable securities having investment grade credit-ratings, assigned by international credit-rating agencies. As of June 30, 2024, we do not hold marketable securities.

On each reporting date, we consider the risk of expected credit loss on bank deposits and marketable securities, if any, including the hypothetical impact arising from the probability of default, which is considered in conjunction with the expected loss caused by default by banks or securities with similar credit-ratings and attributes. In line with previous periods, this assessment did not reveal a material impairment loss, and accordingly no provision for expected credit loss has been recognized.

### ***Equity Risk***

We are exposed from the development in our share price, when remeasuring derivative liabilities at fair value.

Derivative liabilities relate to the foreign currency conversion option embedded in the convertible notes and are measured at fair value through profit or loss. Fair value cannot be measured based on quoted prices in active markets, or other observable input, and accordingly, derivative liabilities are measured by using the Black-Scholes option pricing model, where the pricing is exposed from changes in our share price. Sensitivity analysis over derivative liabilities is disclosed in Note 10, "Financial Assets and Liabilities."

