PRESS RELEASE

# Ascendis Pharma A/S Reports First Quarter 2022 Financial Results

– *Phase 3 PaTHway Trial top-line results demonstrated potential of TransCon™ PTH to become the first parathyroid replacement therapy for adults with hypoparathyroidism*

– *TransCon PTH U.S. FDA regulatory submission on track for Q3 and EU MAA for Q4 2022*

– *Increasing uptake of SKYTROFA® (lonapegsomatropin-tcgd), with more than 1,200 unique patient prescriptions processed as of April 29, 2022*

– *Completed U.S. $575 million convertible notes offering strengthening balance sheet to build a sustainable leading global biopharma company*

– *Conference call today at 4:30 pm ET*

**COPENHAGEN, Denmark, May 11, 2022 (GLOBE NEWSWIRE) –** Ascendis Pharma A/S (Nasdaq: ASND) today announced financial results for the first quarter ended March 31, 2022 and provided a business update.

"This continues to be a transformative time for Ascendis as we build on positive Phase 3 data for our first two endocrinology rare disease programs – TransCon hGH and TransCon PTH - and our first product approval and launch," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "2022 marks a key transition point for Ascendis. We have all the elements of success in place – a proven technology and algorithm for product innovation, the right capabilities, and a strong balance sheet that allows us to deliver on all components of our Vision 3x3 as we work to achieve sustainable growth through multiple approaches and long-term profitability."

**Company Highlights & Progress**

- TransCon hGH:
    - TransCon hGH is commercially available in the U.S. under the brand name SKYTROFA (lonapegsomatropin-tcgd) as the only FDA-approved once-weekly treatment for 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. As of April 29, 2022, 1,231 unique patient prescriptions for SKYTROFA have been written by 404 prescribers and processed through Ascendis' patient hub called the Ascendis Signature Access Program. Nearly 50% of the prescribers have written prescriptions for more than one patient.
    - Coverage for SKYTROFA continues to increase, with approximately 45% of U.S. lives covered at the end of April per MMIT, compared to approximately 36% at the end of February.

- In January 2022, the Company received marketing authorization for Lonapegsomatropin Ascendis Pharma (developed under the brand name of TransCon hGH) in the European Union 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.
- On track for FDA protocol submission during the second quarter of 2022 to evaluate TransCon hGH for Turner Syndrome.
- The ongoing conflict in the region surrounding Ukraine, Russia, and Belarus has impacted our ability to conduct clinical trials or to treat and evaluate patients there. As a result, we have shifted focus for our foresiGHt Trial in adult growth hormone deficiency to other countries and are now targeting completion of enrollment during the fourth quarter of 2022.

- TransCon PTH:
  - On March 13, 2022, we announced top-line data from a randomized, double-blind, placebo-controlled Phase 3 Trial (PaTHway) of TransCon PTH in adults with hypoparathyroidism (HP), demonstrating statistically significant improvement with TransCon PTH compared to control for the primary composite endpoint and all key secondary endpoints. As of May 1, 2022, all 79 patients continued in the open-label extension portion of the PaTHway Trial.
  - On track for planned U.S. NDA submission during the third quarter of 2022 and expected EU MAA submission during the fourth quarter of 2022.
  - After more than two years of treatment in the open-label extension portion of the PaTH Forward Trial, as of May 1, 2022, 57 out of 59 original subjects continue in the trial.
  - In April 2022, enrollment completed in the Phase 3 PaTHway Japan Trial. Top-line results expected later this year.
- TransCon CNP:
  - Top-line data from the ACcomplisH Trial, a Phase 2 randomized, double-blind, placebo-controlled clinical trial in North America, Europe, and Oceania in children ages 2-10 years with achondroplasia are expected in the fourth quarter of 2022.
- TransCon TLR7/8 Agonist:
  - Enrollment continues in transcendIT-101, a Phase 1/2 study of TransCon TLR7/8 Agonist with or without pembrolizumab in patients with advanced or metastatic solid tumors. We expect top-line monotherapy and combo-therapy dose escalation data during the third quarter of 2022.
- TransCon IL-2 β/γ:
  - The Phase 1/2 IL-βelieγe Trial evaluating TransCon IL-2 β/γ monotherapy in patients with locally advanced or metastatic solid tumors continues to enroll patients. Top-line data are expected in the fourth quarter of 2022.
  - On track to dose the first patient in checkpoint combination dose-escalation arm of the IL-βelieγe Trial in the second quarter of 2022.
- TransCon TLR7/8 Agonist and TransCon IL-2 β/γ Combination:
  - We plan to submit an IND or similar for Phase 2 cohort expansion for TransCon TLR7/8 Agonist and TransCon IL-2 β/γ during the fourth quarter of 2022.

- Ended the first quarter of 2022 with cash, cash equivalents, and marketable securities totaling €1,065 million.

**First Quarter 2022 Financial Results**

Total revenue for the first quarter was €6.8 million, an increase of €6.1 million compared to €0.7 million in the same quarter of 2021. Revenues for the first quarter include U.S. commercial SKYTROFA sales, sales of clinical supplies, rendering of services, and recognition of internal profit from the license agreements with VISEN. The increase in revenue was primarily attributable to the €1.9 million commercial SKYTROFA sales, and €3.9 million higher sales of clinical supply to VISEN compared to the same period last year.

Research and development (R&D) costs for the first quarter were €83.2 million compared to €88.1 million during the same period in 2021. Following FDA approval of SKYTROFA (lonapegsomatropin-tcgd) in August 2021, commercial manufacturing is recognized as inventory while such costs were recognized as R&D costs prior to the FDA approval.

Selling, general, and administrative (SG&A) expenses for the first quarter were €47.4 million compared to €37.2 million during the same period in 2021. Higher SG&A expenses were primarily due to an increase in commercial and administrative personnel costs as well as an increase in commercial costs.

Net loss of associate was €4.9 million in the first quarter, compared to a net profit of €28.1 million during the same period in 2021.

Net finance income was €7.6 million in the first quarter compared to €33.6 million in the same period in 2021.

For the first quarter of 2022, Ascendis Pharma reported a net loss of €125.5 million, or €2.21 per share (basic and diluted) compared to a net loss of €62.8 million, or €1.17 per share (basic and diluted) for the same period in 2021.

As of March 31, 2022, Ascendis Pharma had cash, cash equivalents, and marketable securities totaling €1,065 million compared to €790 million as of December 31, 2021. As of March 31, 2022, Ascendis Pharma had 56,958,391 ordinary shares outstanding.

**Conference Call and Webcast Information**

Ascendis Pharma will host a conference call and webcast today at 4:30 pm Eastern Time (ET) to discuss its first quarter 2022 financial results. Details include:

| | |
|---|---|
| **Date** | Wednesday, May 11, 2022 |
| **Time** | 4:30 p.m. Eastern Time/1:30 p.m. Pacific Time |
| **Dial In (U.S.)** | 844-290-3904 |
| **Dial In (International)** | 574-990-1036 |
| **Access Code** | 6876568 |

A live webcast of the conference call will be accessible from the Investors and News section of the Ascendis Pharma website at www.ascendispharma.com. A replay of the webcast will be available on this website shortly after conclusion of the event for 30 days.

**About Ascendis Pharma A/S**

Ascendis Pharma is applying its innovative platform technology to build a leading, fully integrated, global biopharmaceutical company focused on making a meaningful difference in patients' lives. Guided by its core values of patients, science and passion, the company uses its TransCon technologies to create new and potentially best-in-class therapies. Ascendis is headquartered in Copenhagen, Denmark, and has additional facilities in Heidelberg and Berlin, Germany; Palo Alto and Redwood City, California; and Princeton, New Jersey. Please visit www.ascendispharma.com to learn more.

**Forward-Looking Statements**

This press release contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, included in this press release regarding Ascendis' future operations, plans and objectives of management are forward-looking statements. Examples of such statements include, but are not limited to, statements relating to (i) the timing of top-line results from the PaTHway Japan Trial, the ACcomplisH Trial, the transcendIT-101 Trial and the Phase 1/2 IL-βelieγe Trial, (ii) the timing of patient dosing in the combination dose-escalation arm of the IL-βelieγe Trial, (iii) Ascendis' expectations regarding the strength of 2022 and its ability to deliver on components of Vision 3x3, (iv) whether Ascendis is able to achieve sustainable growth and long-term profitability, (v) whether Ascendis has the elements of success in place, including proven technology and algorithm for innovation, the right capabilities, and a strong balance sheet, (vi) Ascendis' expectations regarding the timing of its regulatory submissions, applications, protocols, clinical trials and the results thereof, (vii) Ascendis' expectations regarding the regarding the potential for TransCon PTH to become the first parathyroid replacement therapy for adults with hypoparathyroidism, (viii) Ascendis' expectations regarding the ability of its immuno-oncology programs to transform care for cancer patients, (ix) Ascendis' expectations regarding its ability to apply its technology platform and algorithm for product innovation to develop highly differentiated product candidates for unmet medical needs, (x) Ascendis' ability to apply its platform technology to build a leading, fully integrated biopharma company, and (xi) Ascendis' use of its TransCon technologies to create new and potentially best-in-class therapies. Ascendis may not actually achieve the plans, carry out the intentions or meet the expectations or projections disclosed in the forward-looking statements and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, intentions, expectations and projections disclosed in the forward-looking statements. Various important factors could cause actual results or events to differ materially from the forward-looking statements that Ascendis makes, including the following: dependence on third party manufacturers and distributors to supply TransCon hGH, the SKYTROFA® Auto-Injector and other study drug for commercial sales in the U.S. and clinical studies; unforeseen safety or efficacy results in its oncology programs, TransCon hGH, TransCon PTH and TransCon CNP or other development programs; unforeseen expenses related to commercialization of lonapegsomatropin-tcgd in the U.S. and the further development of TransCon hGH, expenses related to the development and potential commercialization of its oncology programs, TransCon hGH, TransCon PTH and TransCon CNP or other development programs; unforeseen selling, general and administrative expenses, other research and development expenses and Ascendis' business generally; delays in the development of its oncology programs, TransCon hGH, TransCon PTH and TransCon CNP or other development programs related to manufacturing, regulatory requirements, speed of patient recruitment or other unforeseen delays; uncertainty regarding market-size estimates, Ascendis' profitability, and consumer demand for Ascendis' current or future products and product candidates; dependence on third

party manufacturers to supply study drug for planned clinical studies; Ascendis' ability to obtain additional funding, if needed, to support its business activities and the effects on its business from the worldwide COVID-19 pandemic. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to Ascendis' business in general, see Ascendis' Annual Report on Form 20-F filed with the U.S. Securities and Exchange Commission (SEC) on March 2, 2022 and Ascendis' other future reports filed with, or submitted to, the SEC. Forward-looking statements do not reflect the potential impact of any future licensing, collaborations, acquisitions, mergers, dispositions, joint ventures, or investments that Ascendis may enter into or make. Ascendis does not assume any obligation to update any forward-looking statements, except as required by law.

FINANCIAL TABLES FOLLOW

**Ascendis Pharma A/S**
**Consolidated Statements of Profit or Loss and Comprehensive Income / (loss)**
**(In EUR'000s, except share and per share data)**

| | Three Months ended March 31, | |
|---|---|---|
| | **2022** | **2021** |
| Revenue | 6,828 | 746 |
| Cost of sales | 4,246 | - |
| **Gross profit / (loss)** | **2,582** | **746** |
| Research and development costs | 83,193 | 88,149 |
| Selling, general and administrative expenses | 47,418 | 37,247 |
| **Operating profit / (loss)** | **(128,029)** | **(124,650)** |
| Share of profit / (loss) of associate | (4,873) | 28,106 |
| Finance income | 13,044 | 34,430 |
| Finance expenses | 5,399 | 869 |
| **Profit / (loss) before tax** | **(125,257)** | **(62,983)** |
| Tax on profit / (loss) for the period | (241) | 191 |
| **Net profit / (loss) for the period** | **(125,498)** | **(62,792)** |
| Attributable to owners of the Company | (125,498) | (62,792) |
| Basic and diluted earnings / (loss) per share | € (2.21) | € (1.17) |
| Weighted average number of shares used for calculation (basic and diluted) | 56,720,063 | 53,759,952 |
| **Net profit / (loss) for the period** | **(125,498)** | **(62,792)** |
| **Other comprehensive income / (loss)** | | |
| *Items that may be reclassified subsequently to profit or loss:* | | |
| Exchange differences on translating foreign operations | 425 | 1,842 |
| **Other comprehensive income / (loss) for the period, net of tax** | **425** | **1,842** |
| **Total comprehensive income / (loss) for the period, net of tax** | **(125,073)** | **(60,950)** |
| Attributable to owners of the Company | (125,073) | (60,950) |

**Ascendis Pharma A/S**
**Consolidated Statements of Financial Position**
**(In EUR'000s)**

| | March 31, 2022 | December 31, 2021 |
|---|---|---|
| **Assets** | | |
| **Non-current assets** | | |
| Intangible assets | 5,161 | 5,272 |
| Property, plant and equipment | 127,678 | 126,049 |
| Investment in associate | 35,023 | 38,345 |
| Other receivables | 1,822 | 1,808 |
| Marketable securities | 86,487 | 107,561 |
| | **256,171** | **279,035** |
| **Current assets** | | |
| Inventories | 92,436 | 75,405 |
| Trade receivables | 5,808 | 2,200 |
| Income tax receivable | 1,072 | 893 |
| Other receivables | 15,071 | 20,093 |
| Prepayments | 27,994 | 25,231 |
| Marketable securities | 223,055 | 235,797 |
| Cash and cash equivalents | 755,643 | 446,267 |
| | **1,121,079** | **805,886** |
| **Total assets** | **1,377,250** | **1,084,921** |
| **Equity and liabilities** | | |
| **Equity** | | |
| Share capital | 7,649 | 7,646 |
| Distributable equity | 665,167 | 875,989 |
| **Total equity** | **672,816** | **883,635** |
| **Non-current liabilities** | | |
| Borrowings | 464,736 | 97,966 |
| Derivative liabilities | 141,379 | - |
| Contract liabilities | 2,964 | 2,964 |
| | **609,079** | **100,930** |
| **Current liabilities** | | |
| Borrowings | 8,926 | 6,995 |
| Contract liabilities | 265 | 2,601 |
| Trade payables and accrued expenses | 70,683 | 59,417 |
| Other liabilities | 12,536 | 29,952 |
| Income taxes payable | 499 | 198 |
| Provisions | 2,446 | 1,193 |
| | **95,355** | **100,356** |
| **Total liabilities** | **704,434** | **201,286** |
| **Total equity and liabilities** | **1,377,250** | **1,084,921** |

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