PRESS RELEASE

# Ascendis Pharma A/S Reports Full Year 2021 Financial Results and Provides a Business Update

- *Top-line results from PaTHway, a Phase 3 randomized study evaluating the safety, tolerability, and efficacy of TransCon™ PTH in adult hypoparathyroidism (HP) patients on track for this quarter*
- *Demand for SKYTROFA® (lonapegsomatropin-tcgd), the only FDA-approved once-weekly somatropin, continues to grow in the U.S.*
- *Conference call today at 4:30 pm ET*

**COPENHAGEN, Denmark, March 2, 2022 (GLOBE NEWSWIRE) –** Ascendis Pharma A/S (Nasdaq: ASND) today announced financial results for the full year ended December 31, 2021 and provided a business update.

"2021 was an extraordinary year for Ascendis, and we expect that 2022 will be even stronger. The regulatory approvals of TransCon hGH in the U.S. and Europe were key milestones in achieving our Vision 3x3," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer.

"By progressing towards our Vision 3x3, we believe we are moving towards becoming a viable, sustainable, and profitable biopharmaceutical company. We estimate that our first therapeutic area of endocrinology rare disease alone represents a combined US$10 billion global market potential. On top of that, we have a highly differentiated oncology pipeline, and we plan to add a third therapeutic area," continued Mr. Mikkelsen.

## Corporate 2021 Highlights & Anticipated 2022 Milestones

- TransCon hGH:
  - In fall-2021, the Company commercially launched in the U.S., TransCon hGH under the brand name of SKYTROFA (lonapegsomatropin-tcgd), 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 February 28, 2022, 708 SKYTROFA prescriptions were written by approximately 263 prescribers, including 44% writing prescriptions for multiple patients.
  - 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.

- Plan to submit a protocol to the FDA to evaluate TransCon hGH for Turner Syndrome in the second quarter of 2022. The Company expects to evaluate higher doses of TransCon hGH and daily growth hormone for Turner Syndrome compared to those doses for pediatric or adult GHD.
- The ongoing conflict in the region surrounding Ukraine and Russia has impacted our ability to continue clinical trial activities in those countries. While this may affect our timelines for the foresiGHt Trial, our Phase 3 trial in adult growth hormone deficiency, there is currently no material impact to our business from this situation.

- TransCon PTH:
  - In November 2021, the Company announced top-line results from Week-84 of the Company's Phase 2 PaTH Forward Trial evaluating the safety, tolerability, and efficacy of TransCon PTH in adults with HP. The week 84 data showed that subjects treated with TransCon PTH had both mean serum calcium levels and urinary calcium excretion that remained stable and in the normal range and that all subjects continued to be free from taking active vitamin D and 93% were taking less than 600 mg/day of calcium supplements.
  - After two years of treatment in the open label extension portion of the PaTH Forward Trial, as of February 22, 2022, 57 out of 59 original subjects continued in the trial.
  - Top-line results from PaTHway, a Phase 3 randomized, double-blind placebo-controlled clinical trial in North America and Europe, evaluating the safety, tolerability, and efficacy of TransCon PTH in adults with HP, are expected in the first quarter of 2022.
  - If the Phase 3 Trial results are positive, the Company plans to submit a New Drug Application to the FDA during the third quarter of 2022 and expects to submit a Marketing Authorisation Application to the European Medicines Agency during the fourth quarter of 2022.
  - Top-line results from PaTHway Japan, a single-arm Phase 3 trial of TransCon PTH in a minimum of 12 Japanese subjects with HP are expected in the third quarter of 2022.
  - Initiation of a pediatric HP program is planned for the fourth quarter of 2022.
- TransCon CNP:
  - In December 2021, the Company announced completed enrollment in the ACcomplisH Trial, a Phase 2 randomized, double-blind, placebo-controlled clinical trial in North America, Europe, and Oceania in subjects with achondroplasia (ages 2–10). Forty-two percent of subjects enrolled in the ACcomplisH Trial are between the ages of 2 and 5 years old.
  - Top-line data from the ACcomplisH Trial are expected in the fourth quarter of 2022.
  - The Company plans to file an Investigational New Drug (IND) application or similar for the ACcomplisH Infants Trial in subjects with achondroplasia (ages 0–2) during the second quarter of 2022.
- TransCon TLR7/8 Agonist:
  - In December 2021, the Company announced initial first-in-human results from transcendIT-101, a Phase 1/2 study of TransCon TLR7/8 Agonist with or without pembrolizumab in patients with advanced or metastatic solid tumors, which demonstrated early signs of clinical activity in three out of three efficacy-evaluable cancer patients treated with TransCon TLR7/8 Agonist as monotherapy or in combination with pembrolizumab.

    - transcendIT-101 top-line data from monotherapy and combo-therapy dose escalation expected in the third quarter of 2022.

- TransCon IL-2 β/γ:
    - In the third quarter of 2021, the Company submitted an investigational new drug application and initiated IL-βelieγe ("I'll Believe") Trial, a Phase 1/2 clinical trial to evaluate TransCon IL-2 β/γ in patients with advanced cancer.
    - Top-line monotherapy data from the IL-βelieγe Trial are expected in the fourth quarter of 2022.
- TransCon TLR7/8 Agonist and TransCon IL-2 β/γ Combination:
    - The Company plans 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.
- In November 2021, the Company's shareholders elected Rafaèle Tordjman, Founder & CEO of Jeito Capital, to the board.
- Ended 2021 with cash, cash equivalents, and marketable securities totaling €789.6 million.

**Full Year 2021 Financial Results**

Total revenue for 2021 was €7.8 million compared to €7.0 million in 2020. Revenues for 2021 include U.S. SKYTROFA sales, and license, clinical supply and services provided to third parties, primarily VISEN Pharmaceuticals.

Research and development (R&D) costs for 2021 were €295.9 million compared to €260.9 million in 2020. Higher R&D costs in 2021 reflect continued advancement of development programs and an increase in personnel-related costs to support them.

Selling, general, and administrative (SG&A) expenses for 2021 were €160.2 million compared to €76.7 million in 2020. Higher SG&A expenses were primarily due to an increase in commercial and administrative personnel as well as an increase in commercial and IT costs.

Net profit of associate was €12.0 million for 2021, compared to a net loss of €9.5 million for 2020. For 2021, the net profit of associate included a non-cash gain of €42.3 million as a result of a financing round in VISEN.

Net finance income was €55.8 million in 2021 compared to a net expense of €79.0 million in 2020.

For the full year 2021, Ascendis Pharma reported a net loss of €383.6 million, or €7.00 per share (basic and diluted) compared to a net loss of €419.0 million, or €8.28 per share (basic and diluted) for the same period in 2020.

As of December 31, 2021, Ascendis Pharma had cash, cash equivalents, and marketable securities totaling €789.6 million compared to €834.1 million as of December 31, 2020. As of December 31, 2021, Ascendis Pharma had 56,937,682 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 full year 2021 financial results. Details include:

| | |
|---|---|
| **Date** | Wednesday, March 2, 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** | 2009938 |

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 PaTHway, (ii) the demand for SKYTROFA, (iii) Ascendis' expectations regarding the strength of 2022, (iv) whether Ascendis is moving towards becoming a viable, sustainable, and profitable biopharmaceutical company, (v) the estimated market potential of Ascendis' first therapeutic area of endocrinology rare disease, (vi) Ascendis' plan to add a third therapeutic area, (vii) Ascendis' plan to submit a protocol to FDA to evaluate TransCon hGH for Turner Syndrome in the second quarter of 2022, (viii) Ascendis' expectations to evaluate higher doses of TransCon hGH and daily growth hormone for Turner Syndrome compared to those doses for pediatric or adult GHD, (ix) Ascendis' expectations regarding the timing of its applications, protocols, clinical trials and the results thereof, (x) Ascendis' ability to apply its platform technology to build a leading, fully integrated biopharma company, and (xii) 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 10, 2021 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.

*Ascendis, Ascendis Pharma, the Ascendis Pharma logo, the company logo, TransCon, and SKYTROFA are trademarks owned by the Ascendis Pharma Group. © March 2022 Ascendis Pharma A/S.*

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)**

| | Year Ended December 31, | |
|---|---|---|
| | **2021** | **2020** |
| Revenue | 7,778 | 6,953 |
| Cost of sales | 3,523 | - |
| **Gross profit** | **4,255** | **6,953** |
| | | |
| Research and development costs | 295,867 | 260,904 |
| Selling, general and administrative expenses | 160,180 | 76,669 |
| **Operating profit / (loss)** | **(451,792)** | **(330,620)** |
| | | |
| Share of profit / (loss) of associate | 12,041 | (9,524) |
| Finance income | 59,718 | 1,812 |
| Finance expenses | 3,911 | 80,842 |
| **Profit / (loss) before tax** | **(383,944)** | **(419,174)** |
| | | |
| Tax on profit / (loss) for the year | 367 | 219 |
| **Net profit / (loss) for the year** | **(383,577)** | **(418,955)** |
| | | |
| Attributable to owners of the Company | (383,577) | (418,955) |
| | | |
| Basic and diluted earnings / (loss) per share | € (7.00) | € (8.28) |
| Weighted average number of shares used for calculation (basic and diluted) | 54,771,763 | 50,616,528 |
| | | |
| **Net profit / (loss) for the period** | **(383,577)** | **(418,955)** |
| **Other comprehensive income / (loss)** | | |
| *Items that may be reclassified subsequently to profit or loss:* | | |
| Exchange differences on translating foreign operations | 3,855 | (42) |
| **Other comprehensive income / (loss) for the year, net of tax** | **3,855** | **(42)** |
| | | |
| **Total comprehensive income / (loss) for the year, net of tax** | **(379,722)** | **(418,997)** |
| | | |
| Attributable to owners of the Company | (379,722) | (418,997) |

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

| | December 31, 2021 | December 31, 2020 |
|---|---|---|
| **Assets** | | |
| **Non-current assets** | | |
| Intangible assets | 5,272 | 5,717 |
| Property, plant and equipment | 126,049 | 108,112 |
| Investment in associate | 38,345 | 9,176 |
| Other receivables | 1,808 | 1,375 |
| Marketable securities | 107,561 | 115,280 |
| | **279,035** | **239,660** |
| **Current assets** | | |
| Inventories | 75,405 | - |
| Trade receivables | 2,200 | 387 |
| Income tax receivable | 893 | - |
| Other receivables | 20,093 | 6,957 |
| Prepayments | 25,231 | 13,994 |
| Marketable securities | 235,797 | 134,278 |
| Cash and cash equivalents | 446,267 | 584,517 |
| | **805,886** | **740,133** |
| **Total assets** | **1,084,921** | **979,793** |
| **Equity and liabilities** | | |
| **Equity** | | |
| Share capital | 7,646 | 7,217 |
| Distributable equity | 875,989 | 831,494 |
| **Total equity** | **883,635** | **838,711** |
| **Non-current liabilities** | | |
| Lease liabilities | 97,966 | 85,116 |
| Contract liabilities | 2,964 | - |
| Other liabilities | - | 3,162 |
| | **100,930** | **88,278** |
| **Current liabilities** | | |
| Lease liabilities | 6,995 | 6,859 |
| Contract liabilities | 2,601 | 363 |
| Trade payables and accrued expenses | 59,417 | 21,897 |
| Other liabilities | 29,952 | 23,384 |
| Income tax payables | 198 | 301 |
| Provisions | 1,193 | - |
| | **100,356** | **52,804** |
| **Total liabilities** | **201,286** | **141,082** |
| **Total equity and liabilities** | **1,084,921** | **979,793** |

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