

# PRESS RELEASE

# **Ascendis Pharma Introduces Vision 2030**

Strategic roadmap to achieve blockbuster status for multiple products and expand the Company's engine for future innovation

**COPENHAGEN, Denmark, January 7, 2024 (GLOBE NEWSWIRE)** – Ascendis Pharma A/S (Nasdaq: ASND) today introduced selected 2024 corporate milestones and Vision 2030, its strategic roadmap through 2030. Ascendis President and CEO Jan Mikkelsen will present the update tomorrow, January 8, at the 42<sup>nd</sup> Annual J.P. Morgan Healthcare Conference.

"With unwavering focus on our values of Patients, Science, and Passion, we remain on track to fulfill Vision 3x3, with the approval already of two Endocrinology Rare Disease products and value leadership in the U.S. growth hormone market for SKYTROFA," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "Today we are introducing Vision 2030, our strategic roadmap to attain blockbuster status for multiple products and expand our engine for future innovation."

## **Selected Key Updates**

- TransCon<sup>™</sup> hGH
  (lonapegsomatropin, approved as SKYTROFA<sup>®</sup> in the U.S. and EU)
  - o Following positive topline results from Phase 3 foresiGHt Trial in adult growth hormone deficiency (GHD), plan to submit a supplemental Biologics License Application to FDA in the second quarter of 2024.
  - o Topline results from Phase 2 trial in Turner syndrome expected in the fourth quarter of 2024.
  - o Attained U.S. market value leadership in 2023.
  - o SKYTROFA preliminary unaudited fourth quarter 2023 revenue is expected to be approximately €64 million.
  - o Full year 2024 SKYTROFA revenue expected to be €320 million to €340 million (based on average 2023 exchange rates).
- TransCon PTH

(palopegteriparatide, approved as YORVIPATH® in the EU)

- o First launch planned in Germany in January 2024, leveraging the Company's existing commercial infrastructure.
- o FDA regulatory review for the treatment of adult patients with hypoparathyroidism continues, with a Prescription Drug User Fee Act (PDUFA) date of May 14, 2024; if approved, U.S. commercial launch planned in the third quarter of 2024.

# • TransCon CNP (navepegritide)

- Treatment with TransCon CNP at 100 μg/kg/week for one year in all 57 children with achondroplasia (age 2 to 10 years) in ACcomplisH, demonstrated growth consistent with results from the blinded period and achieved improvements in health-related quality of life and disease impacts assessed with SF-10 and Achondroplasia Child Experience Measure.
- During the third quarter of 2023, submitted Clinical Trial Application (CTA) for infant trial (age 0-2 years).
- o Topline results from the pivotal ApproaCH Trial expected in the fourth quarter of 2024.
- During the fourth quarter of 2024, plan to submit a New Drug Application to FDA for children with achondroplasia (age 2-11 years).
- Week 26 topline data from the COACH Trial (TransCon hGH/TransCon CNP combination) expected in the fourth quarter of 2024.
- O During the fourth quarter of 2024, plan to submit an Investigational New Drug application or similar in adults with achondroplasia.
- Global commercial presence in Endocrinology Rare Disease
  - During the fourth quarter of 2023, entered into an exclusive license agreement with Teijin Limited for TransCon hGH, TransCon PTH, and TransCon CNP in Japan. Phase 3 PaTHway Japan Trial of TransCon PTH completed, and Phase 3 riGHt Trial of TransCon hGH fully enrolled.
  - o VISEN's TransCon hGH Phase 3 and TransCon CNP Phase 2 trials in China completed.
  - Expanding global reach through exclusive sales & distribution agreements with geographic market leaders in International Markets, with three regional agreements signed to date: Specialised Therapeutics Asia Pte Ltd, Er-Kim, and Vector Pharma FZCO.

#### Oncology

- O During the fourth quarter of 2024, expect to complete enrollment in BelieveIT-201, a Phase 2 trial in advanced head and neck squamous cell carcinoma (HNSCC).
- O During the fourth quarter of 2024, plan to provide a clinical update from the Phase 2 portion of indication-specific, dose expansion cohorts in the IL-Believe Trial.

# Ophthalmology

- Creation of Ophthalmology NewCo, financed by institutional investors, expected during the first quarter of 2024.
- Financial Update and Outlook Based on Current Plans
  - o Preliminary unaudited December 31, 2023 cash, cash equivalents, and marketable securities of ~€400 million.
  - o Full year 2024 SKYTROFA revenue expected to be €320 million to €340 million (based on average 2023 exchange rates).
  - Expect to provide YORVIPATH revenue update during 2024.
  - Expect total operating expenses (SG&A and R&D) of approximately €600 million for 2024.
  - Expect to be operating cash flow breakeven on a quarterly basis by the end of 2024.

#### Vision 2030: Ascendis Pharma's 2025–2030 Strategic Roadmap

Achieve blockbuster status for multiple products and expand our engine for future innovation.

- Be the Leading Endocrinology Rare Disease Company
  - Achieve blockbuster status (>\$1B) for TransCon PTH, TransCon hGH, and TransCon CNP through worldwide commercialization
  - Be the leader in growth disorders and hypoparathyroidism, pursuing clinical conditions, innovative life cycle management, and complementary patient offerings
  - Expand pipeline with Endocrinology Rare Disease blockbuster product opportunities
- Create Value in Additional Therapeutic Areas through Innovative Business Models
  - Obtain accelerated approval in oncology with registrational trials ongoing
  - Pursue TransCon product opportunities in >\$5B indications
  - Maximize value creation of these product opportunities through collaboration with therapeutic area market leaders
- Differentiate with Ascendis Fundamentals
  - 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

### Presentation at J.P. Morgan Healthcare Conference on Monday, January 8th

A live webcast of the event will be available via the Investors & News section of the Ascendis Pharma website at <a href="https://investors.ascendispharma.com">https://investors.ascendispharma.com</a>. The presentation will begin at 12:00 p.m. Eastern Time / 9:00 am Pacific Time. A webcast replay will be available for 30 days.

The Company's slides from the J.P. Morgan presentation will be available on the same Investor Relations website at <a href="https://investors.ascendispharma.com">https://investors.ascendispharma.com</a>.

#### About Ascendis Pharma A/S

Ascendis Pharma is applying its innovative TransCon technology platform to build a leading, fully integrated biopharma company focused on making a meaningful difference in patients' lives. Guided by its core values of patients, science and passion, Ascendis uses its TransCon technologies to create new and potentially best-in-class therapies. Ascendis is headquartered in Copenhagen, Denmark and has additional facilities in Germany and the United States. Please visit <u>ascendispharma.com</u> 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) Ascendis' ability to achieve blockbuster status for multiple products and expand its engine for future innovation, (ii) Ascendis' ability

to achieve its 2024 corporate milestones and Vision 2030, (iii) Ascendis' ability to fulfill Vision 3x3, (iv) Ascendis' plan to submit a supplemental Biologics License Application for TransCon hGH in the second quarter of 2024, (v) the timing of topline results from the Phase 2 trial in Turner syndrome, (vi) Ascendis' expectations with respect to SKYTROFA revenue for 2023 and 2024, (vii) Ascendis' plan to launch TransCon PTH in Germany in January 2024, (viii) TransCon PTH's PDUFA date of May 14, 2024, (ix) Ascendis' plans to launch TransCon PTH in the U.S. in the third quarter of 2024, if approved, (x) the timing of topline results from the ApproaCH Trial, (xi) Ascendis' plan to submit a New Drug Application for TransCon CNP for children with achondroplasia, (xii) Ascendis' plan to submit an Investigational New Drug application or similar for TransCon CNP in adults with achondroplasia; (xiii) the timing of topline annualized height velocity data from the COACH Trial, (xiv) Ascendis' ability to expand its global reach through exclusive sales & distribution agreements with geographic market leaders, (xv) Ascendis' expectations with regard to the completion of enrollment in BelieveIT-201, (xvi) Ascendis' plan to provide a clinical update from the Phase 2 portion of indication-specific, dose expansion cohorts in the IL-Believe Trial, (xvii) Ascendis' expectations regarding its financial results and performance in 2023 and 2024, (xviii) Ascendis' Vision 2030, (xix) Ascendis' ability to apply its TransCon technology platform to build a leading, fully integrated biopharma company, and (xx) 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, distributors and service providers for Ascendis' products and product candidates; unforeseen safety or efficacy results in Ascendis' development programs or onmarket products; unforeseen expenses related to commercialization of any approved Ascendis products; unforeseen expenses related to Ascendis' development programs; unforeseen selling, general and administrative expenses, other research and development expenses and Ascendis' business generally; delays in the development of its programs related to manufacturing, regulatory requirements, speed of patient recruitment or other unforeseen delays; Ascendis' ability to obtain additional funding, if needed, to support its business activities; the impact of international economic, political, legal, compliance, social and business factors, including inflation, the effects on its business from the worldwide COVID-19 pandemic and ongoing conflicts such as that in the region surrounding Ukraine and Russia. 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 February 16, 2023 and Ascendis' other future reports filed with, or submitted to, the SEC. Forwardlooking 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.

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