

## PRESS RELEASE

### **One-Year Data from Phase 3 Trial of TransCon™ PTH in Adults with Hypoparathyroidism Presented at ENDO 2023**

- *Oral presentation of results showed sustained improvements through Week 52, as well as safety and tolerability similar to that previously reported for the initial 26-week blinded portion of the Phase 3 PaTHway Trial*
- *At Week 52, 95% of patients treated with TransCon PTH achieved independence from conventional therapy*
- *TransCon PTH treatment improved mean patient-reported disease symptom and health-related quality of life scores, starting at the first scheduled follow up after randomization or after switching from placebo and sustained through Week 52*

**COPENHAGEN, Denmark, June 17, 2023 (GLOBE NEWSWIRE)** – Ascendis Pharma A/S (Nasdaq: ASND) today reported one-year (Week 52) data from its ongoing Phase 3 PaTHway Trial of TransCon PTH in adults with hypoparathyroidism. 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. The data were presented by Bart Clarke, M.D., endocrinologist and Professor of Medicine at the Mayo Clinic (Rochester, MN), during ENDO 2023, the annual meeting of the Endocrine Society being held in Chicago.

“We are very pleased to see sustained improvements in clinical outcomes in this trial, including symptom and health-related quality of life measures, consistent with those reported earlier for the initial 26-week blinded portion,” said Jan Mikkelsen, Ascendis Pharma’s President and Chief Executive Officer. “These clinical and patient-reported data, and the retention of 145 patients in our ongoing clinical trials, reinforces our confidence that TransCon PTH has the potential, if approved, to benefit adults with hypoparathyroidism regardless of disease etiology.”

#### **Methods**

PaTHway is a Phase 3 trial of TransCon PTH with a placebo (PBO)-controlled 26-week blinded portion and a 156-week open-label extension (OLE) portion, designed to evaluate the long-term efficacy and safety of TransCon PTH as a potential hormone therapy for those diagnosed with hypoparathyroidism. Results through Week 52 (26 weeks blinded + 26 weeks OLE) were reported at ENDO 2023. Of the 82 study participants dosed, 79 completed blinded treatment and entered the OLE, and 78 (59 TransCon PTH/TransCon PTH, 19 PBO/TransCon PTH) completed Week 52.

#### **Week 52 Highlights**

- 95% of patients in the OLE (74 out of 78) achieved independence from conventional therapy (defined as no active vitamin D and calcium supplements of  $\leq 600$ mg/day), and none required active vitamin D.

- At Week 52, 81% of participants treated with TransCon PTH achieved both normal serum calcium and independence from conventional therapy.
- With TransCon PTH treatment, mean albumin-adjusted serum calcium levels were maintained within the normal range (8.3–10.6 mg/dL) through Week 52 of the OLE (8.9 mg/dL at Week 52).
- Patient-reported scores on the Hypoparathyroidism Patient Experience Scale (HPES) and SF-36 Health Survey showed sustained improvements in disease-related physical and cognitive symptoms, as well as physical functioning and daily life, starting at the first scheduled follow up after randomization or switching from placebo and sustained through Week 52.
- Bone mineral density (BMD) Z-scores continued to trend toward age- and sex-matched norms with 52 weeks of TransCon PTH treatment.
- TransCon PTH normalized 24-hour urine calcium through Week 52, regardless of initial randomization (placebo or TransCon PTH).
- TransCon PTH continued to be well-tolerated in the Phase 3 open-label extension, with no new safety signal identified. Most TEAEs were mild or moderate (Grades 1-2) and none reported during the open-label extension through Week 52 led to discontinuation of the study drug or trial.

Slides showing the data presented for Week 52 of the Phase 3 PaTHway trial of TransCon PTH in adults with hypoparathyroidism can be viewed on the Ascendis Pharma Investors & News website at <https://investors.ascendis.com>.

### **About Ascendis Pharma A/S**

Ascendis Pharma 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. 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 Germany (Heidelberg, Berlin and Munich) and the United States (Palo Alto and Redwood City, California, and Princeton, New Jersey). Visit [ascendispharma.com](https://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 potential approval of TransCon PTH, (ii) TransCon PTH's ability to benefit adults with hypoparathyroidism regardless of disease etiology, (iii) Ascendis' ability to apply its TransCon technology platform to build a leading, fully integrated, global biopharma company, and (v) 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 its development programs or on-market 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, and 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. 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, and TransCon are trademarks owned by the Ascendis Pharma group. © June 2023 Ascendis Pharma A/S.*

**Investor Contacts:**

Tim Lee  
Ascendis Pharma  
+1 (650) 374-6343  
[tle@ascendispharma.com](mailto:tle@ascendispharma.com)  
[ir@ascendispharma.com](mailto:ir@ascendispharma.com)

**Media Contact:**

Melinda Baker  
Ascendis Pharma  
+1 (650) 709-8875  
[media@ascendispharma.com](mailto:media@ascendispharma.com)

Patti Bank  
ICR Westwicke  
+1 (415) 513-1284  
[patti.bank@westwicke.com](mailto:patti.bank@westwicke.com)