Ascendis Pharma A/S Announces Submission of Biologics License Application (BLA) to FDA for TransCon™ hGH in Pediatric Growth Hormone Deficiency

June 26, 2020 at 8:29 AM EDT

– European marketing application on track for third quarter 2020 submission –

COPENHAGEN, Denmark, June 26, 2020 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company that utilizes its innovative TransCon technologies to address unmet medical needs, today announced the submission of a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) for TransCon hGH (lonapegsomatropin), a long-acting once-weekly prodrug of somatropin (human growth hormone or hGH) for treatment for pediatric growth hormone deficiency (GHD).

"TransCon Growth Hormone is the first product candidate we have taken from idea stage, through multiple Phase 3 clinical trials, and now to BLA submission. We believe the TransCon technology has the potential to address major unmet medical needs and we look forward to continuing the late stage clinical development of our other endocrinology rare disease product candidates, TransCon PTH and TransCon CNP, as well as filing an Investigational New Drug application for our first oncology product candidate later this year," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "Most importantly, we are very grateful to all of the participants and investigators in our TransCon hGH trials, and we look forward to discussions with the FDA in an effort to bring this potential new treatment option to children with GHD."

TransCon hGH is designed to maintain the same mode of action as daily hGH therapies by releasing the same growth hormone molecule, somatropin. Currently, there is no approved long-acting growth hormone treatment on the market in the U.S. or Europe. TransCon hGH has orphan designation for GHD in both the United States and Europe.

Ascendis Pharma plans to submit a Marketing Authorisation Application (MAA) for TransCon hGH to the European Medicines Agency in the third quarter, also for pediatric GHD. Additionally, the company expects to initiate a phase 3 trial with TransCon hGH in pediatric GHD in Japan in the fourth quarter, and a phase 3 trial is ongoing in Greater China through the company's strategic investment in VISEN Pharmaceuticals.

About TransCon™ Technology

TransCon refers to "transient conjugation." The proprietary TransCon platform is an innovative technology designed to create new therapies that optimize therapeutic effect, including efficacy, safety and dosing frequency. TransCon molecules have three components: an unmodified 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 conditions (e.g., pH and temperature) initiate the release of the active, unmodified parent drug in a predictable manner. Because the parent drug is unmodified, its original mode of action may be maintained. TransCon technology is designed to be applied broadly to a protein, peptide or small molecule in multiple therapeutic areas, and to be used systemically or locally.

About Pediatric Growth Hormone Deficiency (GHD)

Pediatric GHD is a serious orphan disease caused when the pituitary gland does not produce enough growth hormone. Children with GHD are not only characterized by short stature, but they also may experience metabolic abnormalities, psychosocial challenges and poor quality of life.

For decades, the standard of care for GHD has been a daily subcutaneous injection of hGH, which improves growth and metabolic effects. For caregivers and patients, the treatment burden with daily injections is high, which may lead to poor adherence and reduced overall treatment outcomes.

About Ascendis Pharma A/S

Ascendis Pharma is applying its innovative platform technology 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, the company utilizes its TransCon™ technologies to create new and potentially best-in-class therapies.

Ascendis Pharma currently has a pipeline of three independent endocrinology rare disease product candidates in clinical development and is advancing oncology as its second therapeutic area of focus. The company continues to expand into additional therapeutic areas to address unmet patient needs.

Ascendis is headquartered in Copenhagen, Denmark, with additional offices in Heidelberg and Berlin, Germany, and in Palo Alto and Redwood City, California.

For more information, please visit www.ascendispharma.com.

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 our 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) our plans to submit a Marketing Authorisation Application for TransCon hGH to the European Medicines Agency in the third quarter 2020 for pediatric GHD, (ii) our plans to initiate a phase 3 trial with TransCon hGH in pediatric GHD in Japan in the fourth quarter 2020, (iii) our ability to apply our platform technology to build a leading, fully integrated biopharma company, (iv) our expectations regarding our ability to create potentially best-in-class therapies and (iii) our product pipeline. We 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 we make, including the following: unforeseen safety or efficacy results in our TransCon hGH, TransCon PTH and TransCon CNP or other development programs; unforeseen expenses related to the development and potential commercialization of TransCon hGH, TransCon PTH and TransCon CNP or other development programs, general and administrative expenses, other research and development expenses and our business generally; delays in the development of TransCon hGH, TransCon PTH and TransCon CNP or other development programs related to manufacturing, regulatory requirements, speed of patient recruitment or other unforeseen delays; dependence on third party manufacturers to supply study drug for planned clinical studies; our ability to obtain additional funding, if needed, to support our business activities and the effects on our business of 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 our business in general, see our current and future reports filed with, or submitted to, the U.S. Securities and Exchange Commission (SEC), including our Annual Report on Form 20-F filed with the SEC on April 3, 2020. Forward-looking statements do not reflect the potential impact of any future in-licensing, collaborations, acquisitions, mergers, dispositions, joint ventures, or investments we may enter into or make. We do 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 2020 Ascendis Pharma A/S.

Investor contact:
Patti Bank
Westwicke Partners
(415) 513-1284
patti.bank@westwicke.com
ir@ascendispharma.com

Media contact:
Ami Knoefler
Ascendis Pharma
(650) 739-9952
ack@ascendispharma.com

Source: Ascendis Pharma