



## Growth Hormone Disorders



## Growth Hormone Deficiency

### Study Design

**SkybriGHT** is a U.S., multicenter, prospective, non-interventional study that aims to generate evidence on long-term effectiveness and safety of SKYTROFA® (lonapegsomatropin-tcgd) in patients with growth hormone disorders under routine clinical care.

**SkyPASS** is a prospective, non-interventional (NIS), postauthorization safety study (PASS) that enrolls patients from centers in the U.S. and Europe that aims to further characterize the potential long-term safety risks in patients treated with lonapegsomatropin-tcgd under real world condition in the post-marketing setting.

### Study Endpoints

#### Primary Outcome Measures:

- Assess the safety of patients treated with lonapegsomatropin-tcgd (incidence of AEs and SAEs)
- Assess the effectiveness of patients treated with lonapegsomatropin-tcgd (near adult height [cm])

#### Secondary Outcome Measures:

- Change in Quality of Life in Short Stature Youth (QoLISSY) scores from baseline through End of Study
- Change in Treatment Satisfaction Questionnaire for Medicine (TSQM-9) scores from baseline through End of Study

#### Primary Outcome Measures:

- Occurrence of neoplasms (benign, malignant and unspecified)
- Occurrence of type 2 diabetes mellitus

#### Secondary Outcome Measures:

- Occurrence of renal, hepatic, immunologic and neurologic adverse events
- Occurrence of medication errors in patients treated with lonapegsomatropin-tcgd
- Insulin-like Growth Factor-1 (IGF-1) response to lonapegsomatropin-tcgd therapy

### Is My Patient Eligible?

#### Inclusion Criteria:

- Patients who are on treatment with SKYTROFA (lonapegsomatropin-tcgd)
- Patients being clinically managed in USA
- Patients with an appropriate written informed consent/assent as applicable for the age of the patient

#### Exclusion Criteria:

- Patients participating in any interventional clinical study

#### Inclusion Criteria:

- Pediatric patients with GHD who are on treatment with lonapegsomatropin-tcgd
- Patients being clinically managed in Europe or the USA
- Appropriate written informed consent/assent as applicable for the age of the patient
- Patients willing to comply with follow-up requirements of the study

#### Exclusion Criteria:

- Patients participating in any interventional clinical trial for short stature
- Patients being treated with a GH or IGF-1 therapy, other than lonapegsomatropin-tcgd, at enrollment
- Patients with closed epiphyses



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### Information to Discuss with Your Patients

- Patients will be followed for a max of 10 years
- Informed consent including auto-injector data and questionnaires will be collected every 12 months and 6 months, respectively, for both child and parent

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### TransCon® Technologies



The TransCon name derives from transient conjugation, our unique ability to temporarily link an inert carrier to a parent drug with known biology to achieve sustained release of an active, unmodified parent drug from a TransCon prodrug.

Our TransCon technologies are designed to combine the benefits of conventional prodrug and sustained release technologies to extend duration of a drug's action in the body. **Our goal is to develop drug candidates based on efficacy, safety, tolerability, and convenience.**

### Sponsor Company Profile

Ascendis Pharma is a global biopharmaceutical company focused on applying our innovative TransCon technology platform to make a meaningful difference for patients. Guided by our core values of Patients, Science, and Passion, and following our algorithm for product innovation, we apply TransCon to develop new therapies that demonstrate the potential to address unmet medical needs. Ascendis is headquartered in Copenhagen, Denmark and has additional facilities in Europe and the United States. Please visit [ascendispharma.com](http://ascendispharma.com) to learn more.