User Driven Development of a New Device for Weekly Growth Hormone Administration in Pediatric Patients

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Background

Ascendis Pharma is developing TransCon Growth Hormone, a once-weekly, sustained-release prodrug of recombinant human growth hormone (hGH; somatropin) for the treatment of growth hormone deficiency (GHD) in children and adults.

Current treatment of growth hormone disorders involves daily injections of hGH over many years. Patients are typically diagnosed and initiated on treatment in childhood; adherence is critically important to treatment outcomes. Factors limiting adherence were addressed by developing an easy-to-use electronic autoinjector for once-weekly TransCon GH, currently in Phase 3 development.

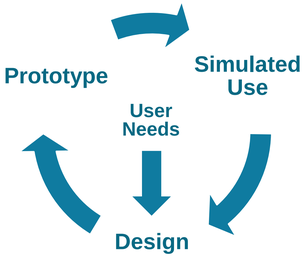
Desirable device characteristics include:

- **Simple operation with few user steps**
- **Weekly, single, low-volume injections (<0.6 mL)**
- **Small needles (31G, 4mm) comparable to daily hGH devices**
- **Room temperature storage**
- **No waste, empty-all design**
- **Long lifespan**
- **Bluetooth® connectivity**

Methods

One hundred and eighteen pediatric subjects, caregivers, health care professionals (HCP) with hGH therapy experience, and other potential users participated in five usability studies conducted in the U.S., Germany, and Denmark.

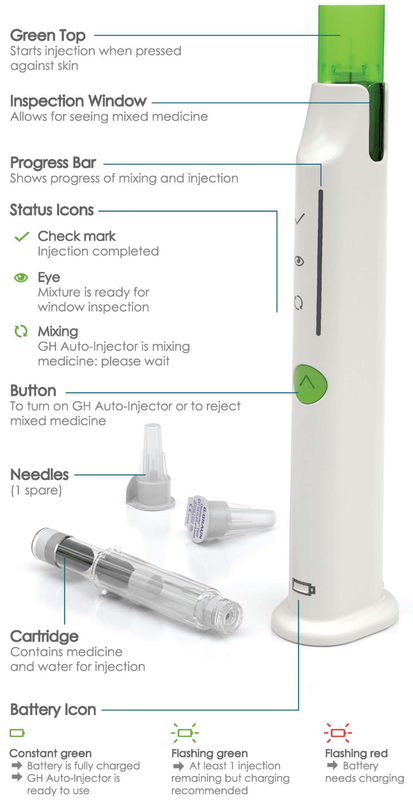
An initial preference study investigated three model concepts with different cartridge loading steps. Six subsequent studies simulated actual usage of the auto-injector prototypes with increasing functionality.



Half of the patients and caregivers were trained on how to use the auto-injector by a moderator using the Quick Reference Guide (QRG). The other half of the patients and caregivers, as well as all HCPs were not trained, but the QRG and Instructions for Use (IFU) were available to them.

All participants completed one or two full use device sequences, including mounting the needle, inserting the cartridge, initiating automatic reconstitution of the lyophilized formulation, auto-injecting into an injection pad, and removing the empty cartridge.

TransCon GH Auto-Injector



Results

Subjects indicated a preference for a cartridge loading into the top of the device with the needle attached.

Subjects appreciated key usability features:

- **Automatic reconstitution of formulation**
- **Feedback on reconstitution progress**
- **No priming or air shot required**
- **Automatic injection upon needle insertion**
- **Hidden needle**
- **Confirmation of completed injection**
- **No handling of used needle**
- **No refrigeration required**
- **Friendly, comfortable form factor**

Conclusion

The TransCon GH Auto-Injector was well accepted, easy to use, and eliminated the need for daily injections, cold storage, priming, air shots, dose settings, and double injections. The electronic device also provided automatic reconstitution and facilitated delivery by auto-injection.

The entire preparation process, from mounting the needle to completing an injection, is designed to take ~5 minutes.
A human factors validation study will be conducted before the auto-injector and cartridge system is included into the ongoing Ascendis Phase 3 heiGHt trial of once weekly TransCon GH.