GenSight Biologics, Inc. Expanded Access Policy for Healthcare Professionals and Patients in the United States (as of June 30, 2018 ; revised on December 8, 2020)

Investigational Medications Covered by this Policy

- GS010 (Lenadogene nolparvovec) for the treatment of Leber Hereditary Optic Neuropathy (LHON)

About Expanded Access

GenSight Biologics is focused on discovering and developing innovative gene therapies for retinal neurodegenerative diseases and central nervous system disorders. Clinical trials form the foundation of our development programs. These trials are designed to assess the safety and efficacy of investigational medicines. Participation in a clinical trial represents the best way, in a controlled setting, to gain access to an investigational medication. Information about ongoing GenSight-sponsored clinical trials can be accessed by consulting https://clinicaltrials.gov/. In some circumstances, a patient may not be able to participate in a clinical trial. Seeking use of an investigational medication under these circumstances is permitted by the FDA and commonly referred to as compassionate use or expanded access. Our policy is intended to comply with U.S. Food and Drug Administration (FDA) requirements for any such use or access.

GenSight's Procedure for Evaluating Requests for Investigational Medication for Expanded Access

- All requests should be sent via e-mail to Dr. Magali Taiel, mtaiel@gensight-biologics.com, and **must originate from the patient's treating physician**; the message header should include the words "REQUEST FOR COMPASSIONATE USE – GS010"
- All requests will be acknowledged within 5 business days of receipt
- In accordance with FDA guidance, compassionate use will be approved only if it does not compromise the supply for ongoing clinical trials.

Criteria for Evaluation

GenSight considers many factors when evaluating a request for expanded access of an investigational medicine, such as (but not limited to) the strength of the clinical data, the benefit-risk profile, the impact on the clinical development program, the phase of development, and probability and timing of regulatory approval.

A patient may be considered for expanded access to an investigational medicine based upon evaluation of the following minimum requirements (and others may apply based on the circumstances):

- There must be adequate human data supporting use of the investigational medication in the treatment of the patient's disease.

- There are no adequate alternative therapies or clinical trials available.
- Sufficient safety and efficacy data must exist for use of the medicine at the time of request. Generally, this will not occur before Phase 3.
- Sufficient information must be made available to make a benefit-risk analysis consistent with the establishment of an expanded access program.
- Sufficient clinical data is available to identify an appropriate patient-specific dose.
- A patient's treating physician and GenSight's Chief Medical Officer both believe there is the potential for the specific patient under consideration to reasonably expect benefit from the treatment.
- Adequate supply exists to support both the ongoing clinical trials and approved expanded access, until and if product becomes commercially available.
- The patient is not eligible or a candidate for a GenSight-sponsored study for the therapy being requested.
- Expanded access will not adversely impact the clinical development program or the regulatory approval process.
- The request must be made by the patient's treating physician. The physician must be appropriately licensed.
- The treating physician must also agree to comply with the safety and monitoring requirements.
- Any condition, in addition to those listed above, that in the opinion of GenSight, may preclude appropriate use of the investigational medicine, will be considered.

As stated, generally, the safety and efficacy data needed to grant expanded access are not available until the initiation of Phase 3 of clinical development. Given the unique nature of individual requests for compassionate use of investigational medications, even if a drug has entered Phase 3 of development there is no guarantee a request will be fulfilled.

GenSight may revise, suspend or terminate the expanded access program at any time.

Each expanded access request requires approval by the Chief Medical Officer, Head of Regulatory Affairs, Head of Manufacturing, and Chief Executive Officer.

Contact Information

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