



Expanded Access & Compassionate Use Policy

Summary | Last updated: April 2026

Our Commitment

Genetix Biotherapeutics is dedicated to developing transformative gene therapies for patients with serious and life-threatening diseases. We believe rigorous clinical trials are the most effective path to demonstrating safety and efficacy. For patients who cannot participate in a trial and have exhausted all available options, Genetix may consider expanded access (compassionate use) requests on a case-by-case basis.

Eligibility Criteria

Requests may be considered when all of the following conditions are met:

- The patient has a serious or life-threatening condition with no comparable or satisfactory alternative therapy available.
- The patient is ineligible for, or otherwise unable to participate in, an active or planned Genetix-sponsored clinical trial.
- Sufficient clinical evidence supports that the potential benefit outweighs the potential risks to the patient.
- Adequate material supply exists without compromising ongoing or planned clinical trials.
- The treating physician has the qualifications, facilities, and capabilities to administer the therapy and monitor adverse events.
- The patient is willing and able to commit to long-term follow-up as required by applicable regulatory authorities.

Important Considerations

Expanded access is generally considered only after initiation of Phase 3 clinical studies, when sufficient safety and efficacy data are available. All expanded access is subject to approval by the applicable regulatory authority (e.g., FDA), an Institutional Review Board or Ethics Committee, and informed patient consent. All decisions are made at the sole discretion of Genetix Biotherapeutics and in accordance with this policy. Genetix cannot guarantee access to any investigational therapy for any individual patient.

How to Request

Requests must be initiated by a licensed physician on behalf of their patient through a Genetix-designated Qualified Treatment Center (QTC). Patients and caregivers are encouraged to first speak with their treating physician about clinical trial eligibility.

Contact Medical Information: medinfo@genetixbiotx.com | 1-833-999-6378

Genetix will acknowledge receipt of a complete request within five (5) business days.