

Abeona Therapeutics

Pre-Approval Access Policy for Investigational Therapies

Our Commitment

Abeona Therapeutics was created with the goal of transforming scientific discoveries into breakthrough therapies for children and adults living with rare and often life-threatening diseases. We understand that success can only be achieved by collaborating closely with the families, researchers, patient groups, physicians, and regulators who share our mission of serving rare disease communities.

Together with these stakeholders, Abeona is evaluating the potential of multiple investigational gene therapies, each at various stages of development in animals (pre-clinical research) and patients with rare disease (clinical trial). Our focus is to advance each potential therapy toward regulatory approval and make them available to those who can benefit from them.

Clinical Trials

[Clinical trials](#) are key research tools for advancing medical knowledge, utilizing innovative strategies to improve patient care. Clinicians conduct these studies in specific patient populations to determine whether a new medicine works and is safe to use in people. Clinical trials are also conducted to find out which treatments or strategies work best for certain illnesses or groups of people. Trials are carefully planned and monitored to answer these scientific questions. Participation in U.S. Food and Drug Administration (FDA) or other relevant regulatory agency accepted clinical trials is the best way to access investigational therapies. At this time, participation in clinical trials is the only way for patients to gain access to investigational therapies under development by Abeona.

Pre-Approval Access Outside of Clinical Trials

We recognize that not all patients will meet the eligibility requirements for participation in clinical trials and as a result, understand that some may seek pre-approval access, also referred to as expanded access or named patient use.

Abeona relies on guidelines provided by the [FDA](#) and other regulatory agencies along with other important factors when evaluating our ability to provide pre-approval access for any of our investigational gene therapies. Key considerations include:

- Whether there is sufficient evidence that the potential benefits to the patient(s) outweigh the potential risks, based on available safety and efficacy data.
- Whether sufficient clinical data is available to identify a recommended dose that might be effective and is reasonably safe.
- Whether there is sufficient clinical data to identify an appropriate dose for use in children, if the request is for a child.
- Whether providing pre-approval access outside of a trial setting will compromise or delay ongoing clinical trials and/or future access by the broader patient community.

- Whether there is adequate supply of the investigational gene therapy to complete ongoing and planned clinical trials.
- Whether Abeona can provide the investigational gene therapy and associated medical care in a fair and equitable manner.
- Whether it is reasonably feasible to provide the investigational gene therapy and any necessary follow up outside of the clinical trial setting.

What to do if you have Questions.

Treating physicians, individuals and/or caregivers interested in learning more about Abeona's investigational gene therapies currently undergoing clinical study can find more information [here](#). For other questions, please contact access@abeonatherapeutics.com. We will acknowledge receipt and respond to inquiries within seven (7) days.

As more information and clinical data becomes available, Abeona may revise our Pre-Approval Access Policy. If you would like to receive any potential updates, please request to be included in update emails by sending a message to access@abeonatherapeutics.com.

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