Disease Nomination Review Process

TIMELINE

December 3, 2021
FNIH disease nomination request launch

January 6, 2022
BGTC explanatory webinar

February 18, 2022
Application close date;
63 BGTC disease nominations received

Late May 2022
Review of all applications complete

June 2022
Selected nominations announced for full application submission

Summer 2022
Clinical trial proposal review

Fall 2022
Disease selection announcement

PROCESS

1 Application Check

- Application is complete
- Nominee meets definition of "rare disease"

2 Merit Review

- 3 reviewers perform independent reviews for each application
- Review teams include:
  - Expertise in disease area/organ system involved
  - Expertise in AAV gene therapies
  - Expertise with clinical trials
- Applications are scored against the criteria established in the disease nomination form
- Each reviewer supplies written narrative summary of strengths/weaknesses
- *NIH Conflict of Interest Policy* (COI) applied to reviewers

3 BGTC Steering Committee Subteam Recommendation

- 3 merit reviews for each application aggregated to a combined score
- Disparities in scoring reconciled by Subteam
- For additional consideration
  - "Portfolio approach" to ensure broad diversity among disease states and potential patient populations participating in clinical trials
  - Strength of criteria indicating clinical trial readiness*
- Recommended applications move to full Steering Committee

4 BGTC Steering Committee Decision

- Subteam recommendations reviewed
- Steering Committee votes on final 12–15 diseases to move to submission of full applications
- Selected diseases announced and Clinical Trials RFP issued

* Criteria include gene target able to be inserted into an AAV vector; sufficient proof of concept and natural history studies; established disease models—at the gene, organ, or full-animal level—able to demonstrate transgene functionality, dose finding studies, and conduct safety and efficacy testing; existence of at least one validated, clinical meaningful outcome measure with suitability for an AAV gene therapy clinical trial; a known patient population affected by the disease and supported by a patient advocacy group; and not commercially viable.