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.