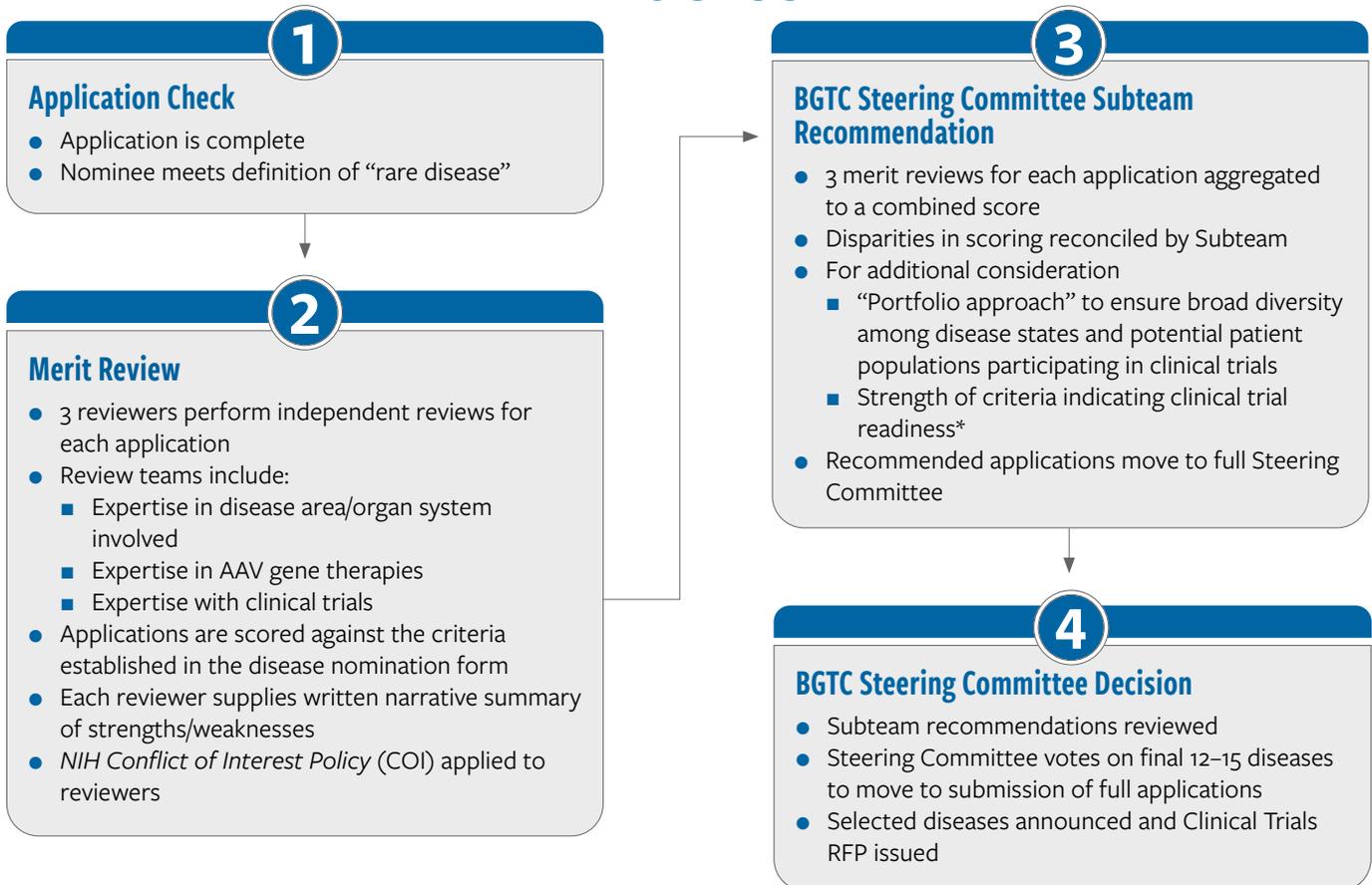


Disease Nomination Review Process

TIMELINE



PROCESS



* 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.