

Advisory Committee on Heritable Disorders in Newborns and Children

Expedited Review Process

Expedited Review – background

- The final step in the process of deciding to recommend adding a new condition to the RUSP involves Committee review and discussion of the evidence review and synthesis and vote(s)
- If the vote does not result in a recommendation to add, there may be specific evidence gaps or other issues that could be addressed in a short time frame, i.e., within a calendar year

Expedited Review – background (cont.)

- The nominators may choose to respond to address issues and submit new evidence and/or other revisions within the one-year time frame
- It is expected that in these cases, the Evidence Review Group could do an expedited review, incorporating new evidence or addressing other revisions such as change of scope without starting the evidence review process over

Expedited Review Process

1. If the committee vote results in not recommending a condition to the RUSP, the chair sends a letter to the nominators summarizing the issues leading to the decision (per current practice, within 60 days of the ACHDNC meeting)
2. Within one year of the chair's letter, nominators may resubmit a re-nomination package for expedited review

Expedited Review Process (cont.)

3. Requests for an expedited review must include responses to the chair's letter and may include additional new evidence or information on other relevant issues
4. The request must outline at least one material change and include supporting data/documents
 - A material change involves a change in scope of the condition nominated and/or substantial new evidence for the nominated condition
 - If there is a change in scope, it is preferable that there is also new evidence provided in support of the nomination

Chair's letter and nomination for expedited review-caveats

- The nomination package resubmission must address committee questions and comments described in the chair's letter to the nominators
 - However, nominators should realize that the chair's letter only includes issues that were raised in the discussion—there maybe other reasons a committee member voted against adding the condition
 - The committee does not want to create expectations that addressing the issues in the letter alone will result in a changed vote for any member

Expedited Review Process (cont.)

5. The chair reviews the re-nomination package and, with input as necessary from ERG and N&P Workgroup, determines if it qualifies as a material change
6. This step is likely to involve ongoing discussion with the nominators and will be performed as expeditiously as is practical for all participants

Expedited Review Process (cont.)

7. If the chair concludes the renomination constitutes a material change, the package will be presented and discussed by the full committee for consideration for an expedited review
8. The committee should vote on whether to move the condition nomination to an expedited review, to be conducted by the ERG
9. If the vote fails, the chair will summarize the issues leading to the decision in a letter to the nomination group, and the condition will return to the list of conditions for future nomination and prioritization

Expedited Review Process (cont.)

- If the chair concludes there is not a material change, the nominators and the rest of the committee will be notified
- The condition will return to the list of conditions for future nomination and prioritization

Expedited Review Process (cont.)

10. If the vote passes, if necessary, the N&P workgroup will prioritize the review considering, other topics in the prioritization queue, in order to determine timelines/deadlines
11. The ERG will follow standard systematic review processes to identify relevant research published since completing the previous review
 - The ERG may find additional new research on issues not included in the original review and committee discussion (and chair's letter) that could impact the decision to recommend the condition be added to the RUSP

Expedited Review Process (cont.)

10. The ERG will work with the technical evaluation panel (TEP) and revises the review to include the new evidence and/or address any revision in scope; this may involve additional modeling
11. When the review is complete, the condition will be scheduled for presentation, discussion and vote for recommendation for inclusion on the RUSP at a regular committee meeting
12. A vote on the condition must be held within 9 months after approval of expedited review

Expedited Review Process – concluding comments

- It must be clear to all that a topic approved for expedited review may still not be recommended for addition to the RUSP
- It is the intent and expectation of the committee that this process will be engaged rarely, for very few nominated conditions—this is not intended to become a routine process for most or many conditions
- The committee will review/evaluate this process after two expedited review requests to identify and implement any appropriate revisions