MGH Center for Child and Adolescent Health Policy

Draft Template for Evidence Reviews for the Advisory Committee on Heritable Disorders and Genetic Diseases in Newborns and Children
September 17, 2007

Background

1) Basic description of the condition itself
    a. Prevalence
    b. Genetics
    c. Natural history
        i. Severity and burden
        ii. Variations in phenotype
2) Rationale for review at this time
    a. Nomination form and consideration by Advisory Committee
    b. Recent changes in treatments and/or screening

Methods

1) Data sources
    a. How they were obtained
    b. Special issues re format/data files and constraints on data or elements
    c. Inclusion/exclusion criteria
        i. Human only
        ii. Exclude case reports
        iii. Review expert consensus documents, as guides, not for abstraction)
    d. Main sources
        i. Peer-reviewed published literature
        ii. Key investigators
        iii. Pharmaceutical companies
        iv. FDA
2) Decision model and development of evidence questions
3) Data abstraction
4) Analyses of (any) raw data (may receive data files from sources that require analysis – or files that may allow for additional analyses)
5) Focus groups of experts (investigators and families) re burden and severity estimates
6) Main questions
    a. Natural history, including variations
    b. Prevalence
    c. Burden and severity
    d. Methods of screening and diagnosis
    e. Treatment effectiveness and variations
        i. Benefits
        ii. Risks
    f. Costs – of screening, of treatment

Results

1) Presentation will follow order and content of main questions
2) Decision analyses/decision model findings

Summary of key findings

What do we not know; what is the level of uncertainty? What new information or studies would help with answering study questions?