



Process to Evaluate Conditions for Inclusion in the Required Newborn Screening Panel

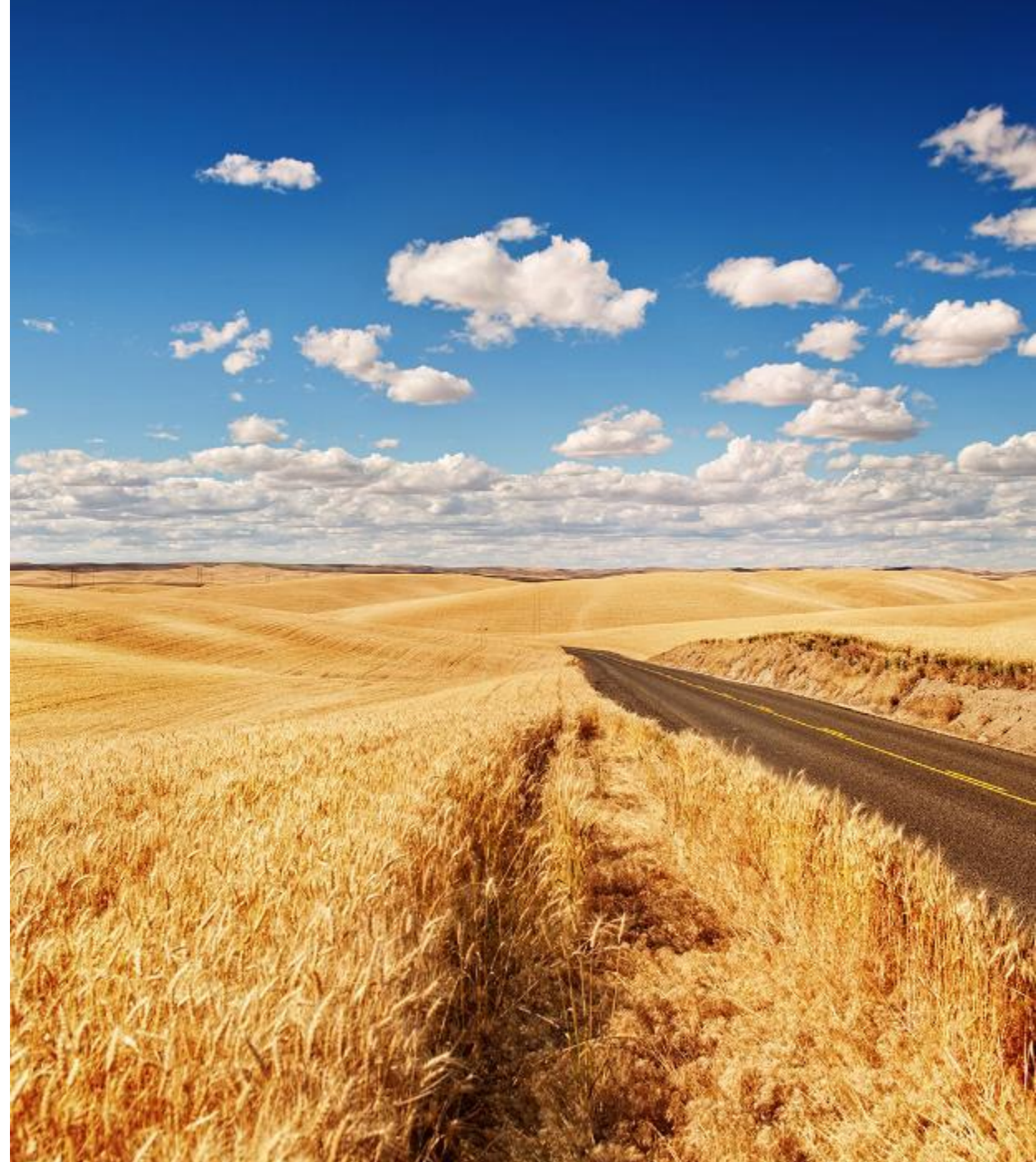
September 21, 2022

Three Guiding Principles



Driven by Evidence

Decision to add a screening test should be driven by evidence. For example, test reliability and available treatment have been scientifically evaluated, and those treatments can improve health outcomes for affected children.



Accessibility

All children who screen positive should have reasonable access to diagnostic and treatment services.



Benefits Outweigh Harms

Benefits of screening for the disease/condition should outweigh harm to families, children and society.



Five Criteria

Available
Screening
Technology

Diagnostic
Testing and
Treatment
Available

Prevention
Potential
and Medical
Rationale

Public
Health
Rationale

Cost-Benefit
/ Cost-
Effectiveness

Available Screening Technology

Sensitive, specific and timely tests are available that can be adapted to mass screening.



Diagnostic Testing and Treatment Available

Accurate diagnostic tests, medical expertise, and effective treatment are available for evaluation and care of all infants identified with the condition.



Prevention Potential and Medical Rationale

The newborn identification of the condition allows early diagnosis and intervention.
Important considerations:

- There is sufficient time between birth and onset of irreversible harm to allow for diagnosis and intervention.
- The benefits of detecting and treating early onset forms of the condition (within one year of life) balance the impact of detecting late onset forms of the condition.
- Newborn screening is not appropriate for conditions that only present in adulthood.



Public Health Rationale

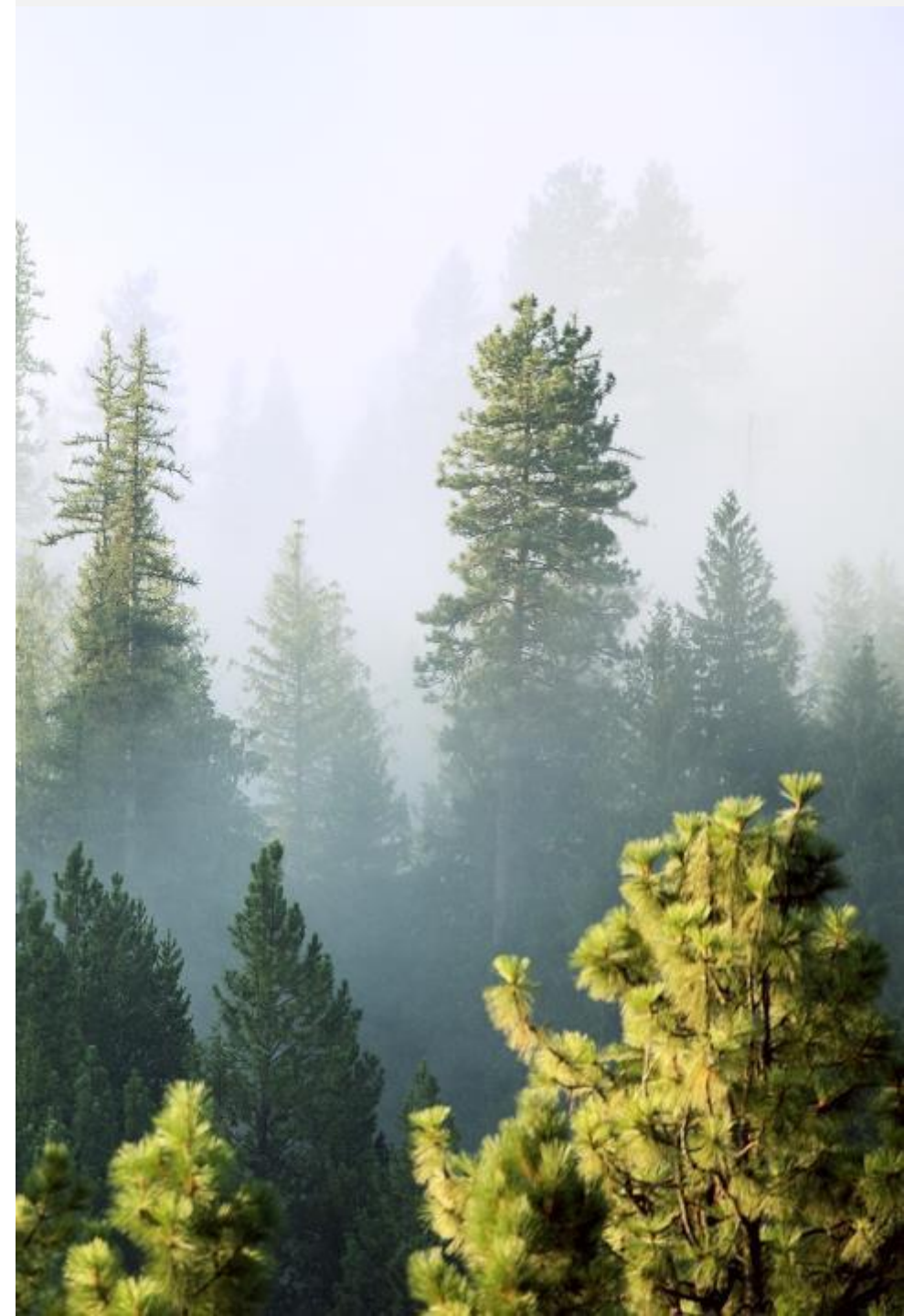
Nature of the condition justifies population-based screening rather than risk-based screening or other approaches.



Cost-benefit / Cost-effectiveness

The outcomes outweigh the costs of screening. All outcomes, both positive and negative, need to be considered in the analysis. Important considerations to be included in economic analyses include:

- The prevalence of the condition among newborns.
- The positive and negative predictive values of the screening and diagnostic tests.
- Variability of clinical presentation by those who have the condition.
- The impact of ambiguous results. For example, the emotional and economic impact on the family and medical system.
- Adverse effects or unintended consequences of screening.



QUESTIONS?