Special Populations & Rare Diseases

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Definitions of Special Populations

- Special Populations
  - Children and the lifecourse approach
  - Older populations
  - Health disparities and the underserved

- Rare Diseases
  - Childhood disease
  - Certain cancers
Themes

- Diseases in the very elderly
  - Do treatments for cancer and cardiovascular disease have a different impact in the elderly, vs. younger adults?

- Natural history of rare diseases
  - Can we define the natural history and clinical characteristics of rare diseases to use as a benchmark for defining benefit from novel therapeutics?

- Rare childhood diseases
  - Healthcare Cost and Utilization Project Kids Inpatient Database (HCUP KID)
    - Largest publicly-available all-payer pediatric inpatient care database in the United States