NAS/IOM Review of Rare Diseases Research and Orphan Products Development - USA

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IOM Review of Rare Diseases Research and Orphan Products Development

- Conduct an independent assessment of the current strategies and incentives for the development of therapies for rare diseases
- Provide recommendations to improve these strategies and incentives and shorten the timeline for development of new treatments and cures.

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- Make recommendations for an integrated national rare disease policy on research and development
- Assess existing strategies to promote research discoveries and development of orphan products to improve the health of people with rare diseases.

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- Examine current public policies relevant to product development for rare diseases, including the
  - Orphan Drug Act,
  - Humanitarian Use Device Exemption,
  - Approaches of the National Institutes of Health and the Food and Drug Administration,
  - Reimbursement policies, and
  - Other legislative and regulatory initiatives.

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- Describe the epidemiology and societal impact of rare diseases and provide an overview of current methods for their prevention, diagnosis, and treatment.
- Describe the strengths and limitations of the current development pathways for new drugs, medical devices, and biologics for rare diseases (taking into account developments in genetic testing) and discuss the special challenges that rare diseases create for research and product regulation.

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- **Enhancing multidisciplinary collaboration and government-university-industry partnerships in basic and translational research;**
- **Expanding public engagement and enhancing the roles of patient organizations;**
- **Facilitating research data and biomaterials collection and dissemination, including the use of bio-repositories and registries;**
- **Strengthening training of investigators;**

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- **Disseminating information to clinicians, patients, and families;**
- **Review of policies and regulations;**
- **Encouraging alternative research financing mechanisms; and**
- **Developing research agendas and coordinating resources and development efforts throughout the product development pathways.**