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PERSONALIZED MEDICINE: Viewing Product Approval through Mechanism of Action vs Disease State

ICORD

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Marlene E. Haffner, MD, MPH

Rare Diseases are Not Rare

- ~ 7000 rare diseases
- 2008 Better knowledge/understanding of molecular medicine
- Better understanding and targeting of drugs
- Personalized medicine

Mechanization of Action

- **How many rare/common diseases have traits that can be treated by drugs with similar mechanism of action**
- **Oncology products**
- **Gleevec®**
- **PTC - 124**

- **PTC – Stop Codon**
- **15% of all genetic diseases have a stop codon mutation**
 - **#s are small; 30,000 patients with CF, 10% with stop codon mutation, 3000 patients**

- **But 2400 Potential Diseases -**
 - **#s in each disease small**
 - **If mode of action proves correct**
 - **2400 clinical development plans ??**

Gleevec®

- **Indications**
 - 10 adult
 - 4 pediatric
- **5 FDA Approvals**
 - CML
 - GIST
 - Dermatofibrosarcoma Protuberans
 - Myelodysplastic Syndrome
 - Systemic Mastocytosis

FDA via Critical path looking to progress/change drug development

- **21st Century current knowledge has need to use 21st Century tools**
- **Is it time to look at mechanism of action when considering drug development?**
- **How to proceed?**