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

**ICORD**

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# 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**

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