

ICORD 2008



**Access to Information
& to Orphan Products:
Industry's View**

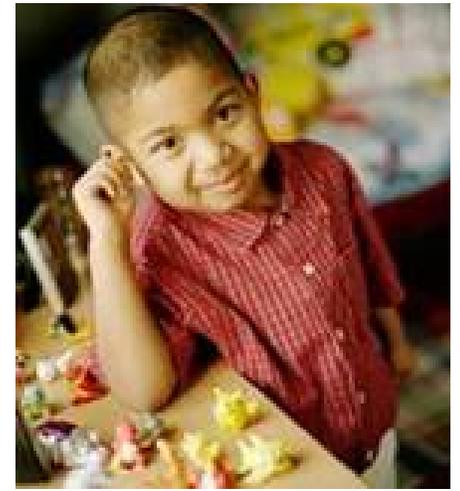
Erik Tambuyzer, Genzyme

25 years of US Orphan Drug Act : How Do We Continue?

- **CONGRATULATIONS** for the pioneers : NORD, NIH and FDA
- Other regions have followed the USA in creating a system to help patients by fostering orphan drug development
- After 8 Years of EU Orphan Medicines Regulation: similar annual number of designations and approvals in EU and USA
- Stakeholders, including industry, are delivering on their promise: more therapies for patients with rare disorders!

Hope

- For patients and their care takers looking forward to the prospect of a treatment
- For children born with treatable rare diseases: the potential to live a normal life

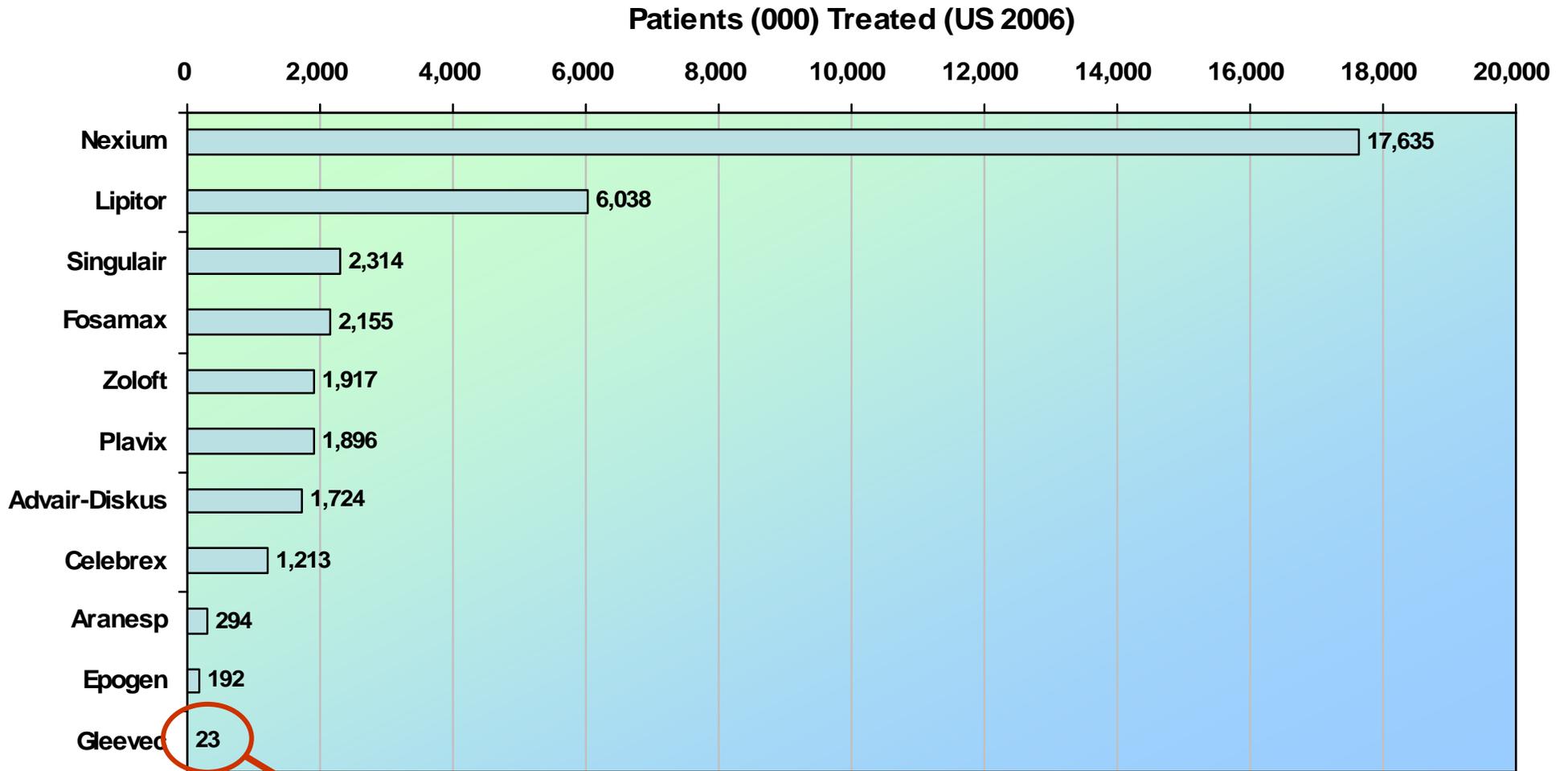


Fear

- For payers afraid of the impact of orphan drug costs on health care budgets
 - High prices?
 - A Tsunami of orphan drugs coming?
 - Salami-slicing? Off-label use?
 - Misuse of the legislations?



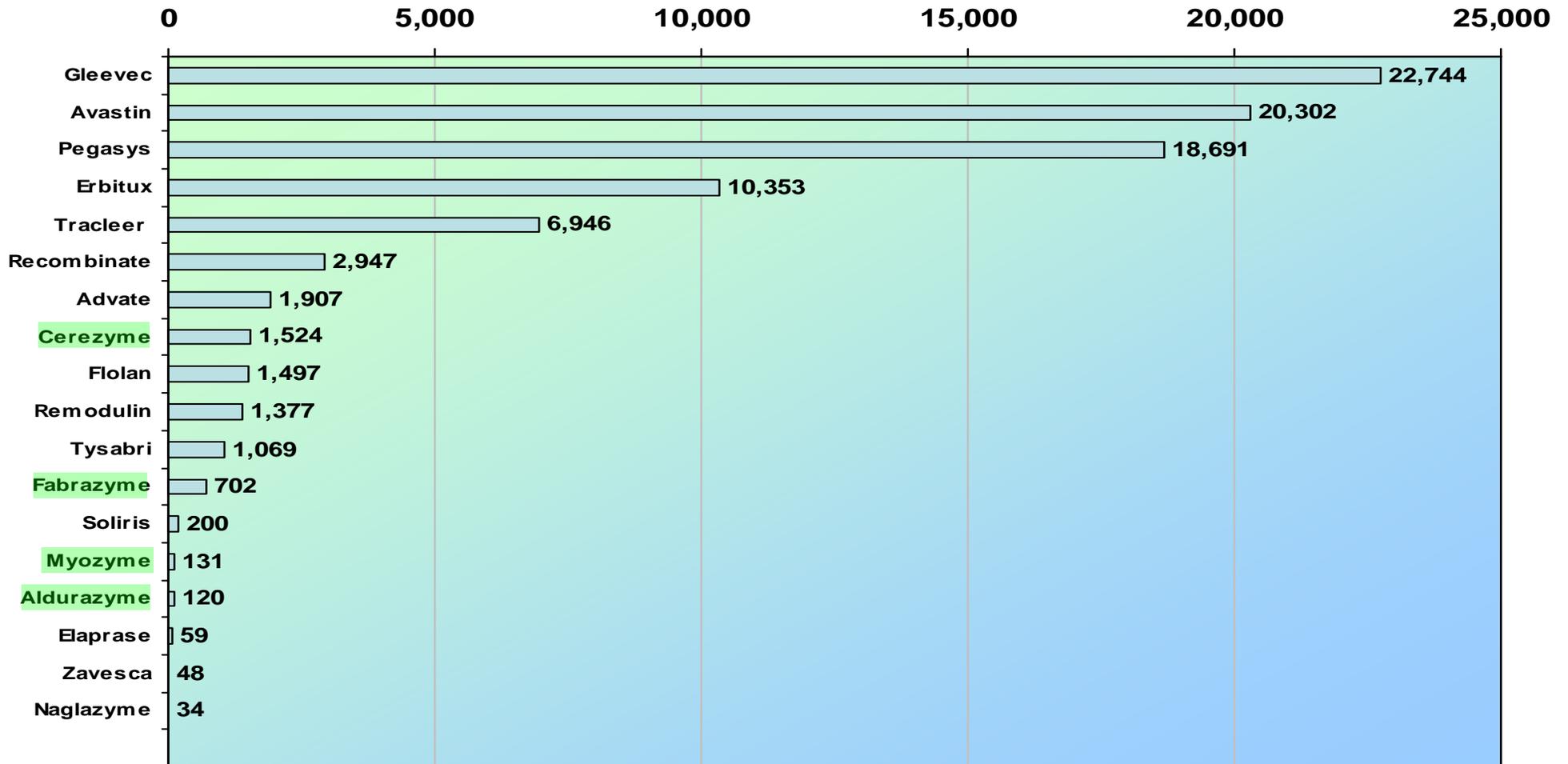
Understanding Rarity



Orphan Drug (<200,000 prevalence in US)

Understanding Rarity

Patients Treated (US 2006)



Address misconceptions with clearer messages from us all

- ◆ Orphan medicines are treatments for life-threatening or serious and chronic diseases
- ◆ Estimated prevalence is not the same as treatable number of patients
- ◆ Common, rare (orphan), ultra-rare (ultra-orphan) diseases: the reality is a **continuum** with research and treatment complexity increasing with rarity
- ◆ There is no avalanche, but rather a steady increase of 10-12 new products annually (EPPOSI workshop Copenhagen, 2007)

Industry makes substantial contributions

- Innovation for unmet medical needs
- A major source of healthcare products, including orphan drugs
- Expanded Access/compassionate use programs
- Wealth & employment creation

But needs to communicate better

**Policy needs: in the US and
EU: from R+R → A+A**

**The focus needs shifting
from “only”**

**Research and Regulatory issues
to
Awareness and Access**

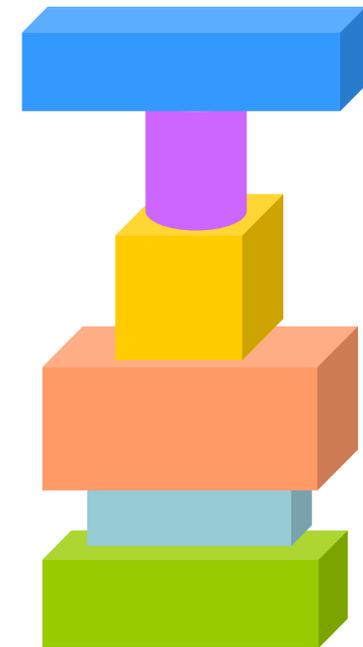
Policy needs in other parts of the world without OD system

Include Awareness and Access issues into the discussions on new legislation regarding orphan drugs?

A broader Healthcare System Building Access & Optimal Patient Care

- Diagnostic infrastructure
- Treatment guidelines
- Centres of excellence
- Registries
- Compassionate use
- Dedicated and “centralized” funding
- Patient network

- Dialogue, Partnership and Transparency





THANK YOU