

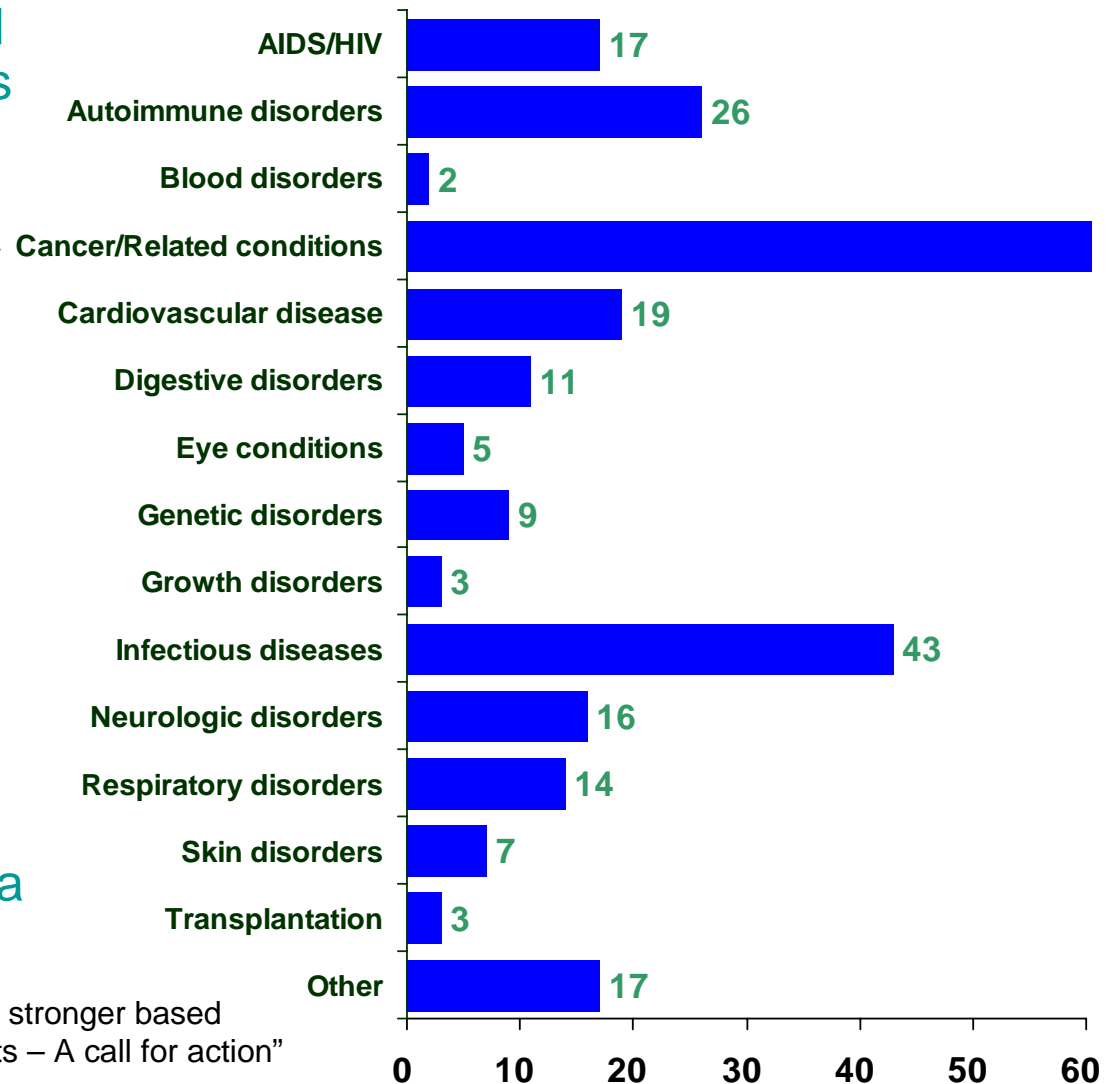


Malattie Rare e Disabilità Roma, 1 Dicembre 2006

Pioneering Targeted Medicine
through Biotech-based
Innovation for Unmet Needs
Dr. Massimo Boriero
Senior Vice President
International Group
Genzyme s.r.l.

Biotechnology in Human Healthcare Today

- 20% of all medicines and 50% of all new medicines in development
- 324 new biotech medicines & vaccines for 150 diseases
- 154 for cancer, 43 for infectious diseases and 26 for auto-immune diseases
- Over 250 million patients with cardiovascular diseases, multiple sclerosis, breast cancer, rare diseases and cancers such as leukemia are being helped



Source: European Commission communication – “A stronger based pharmaceutical industry for the benefit of the patients – A call for action”

Biotech Looks at Disease Causes with New Approaches

- Improving **disease knowledge and diagnosis**
 - Genomics, genetic testing, proteomics, RNA and DNA*
 - Underlying mechanisms of disease*
- Addressing **unmet medical needs**
 - Rare genetic diseases*, cancer*, biosurgery*, HIV/hepatitis treatments, anemia, diabetes, hypercholesterolemia*
- Pioneering **targeted medicine**
 - **Diagnosis linked to therapy***
 - Pharmacogenetics and fewer side-effects*
 - Monoclonal antibody-based biotherapeutics*
- New technologies for the future
 - **Cell therapy and tissue engineering***
 - **Gene therapy***
 - Silencing of and interference with RNA

* Means Genzyme activity

Genzyme's Commitment to Innovation

We seek frontiers



- Serious diseases – unmet needs



We develop and deliver breakthrough therapies and services



- Significant improvement to patients' lives



- Patient-focused
- Variety of technology platforms
- Life-changing standard of care products
- Creating sustainable value

Innovation and Growth go Hand in Hand

- Commitment to **innovation**: orphan drugs, cell therapy, biosurgery, gene therapy, genetics, ...
- Company **culture is key**: core values serve as compass
 - Entrepreneurial
 - Independent
 - Societal input and consensus building
 - Supporting a positive regulatory climate
 - Technology important but not dominant
 - Financial sustainability
 - Global access to our products

Examples of Innovation at Genzyme

- **Enzyme Replacement Therapies**
 - Treatment of Lysosomal Storage Diseases
- **Gene and Cell Therapies**
 - Autologous Chondrocyte Implantation for damaged cartilage
 - Research on gene therapy for rare diseases
 - Gene therapy for intermittent claudication
- **Genetics**
 - **Screening** tests for genetic diseases such as Cystic Fibrosis, but also for rare diseases with therapy available
 - Diagnostic tests for various cancer types

Rare Disorders & Orphan Medicines

- Over 6000 rare disorders, 70-80% genetic, affecting 4-5% of the population
- Innovation in approach and in treatments needed
- Cost of developing medicine not recoverable without economic incentives → industry
- Orphan medicine is for life-threatening or serious/chronic rare disorder → no “parent”
- 30-50% of orphan drugs are derived from biotechnology

Orphan Drug regulations in the USA and the European Union

Purpose:

- To provide **safe and effective** therapies for patients with rare diseases, and
- **Incentives for industry** to develop and market these therapies

Core:

- **Societal values** representing the desire to provide equitable access to therapies independent of the rarity of a disease
- Regulation only for therapies to treat **life-threatening and/or serious & chronic diseases**

Legislations

- **US Orphan Drugs Act** adopted in 1983
- **Japan orphan drugs law** in 1993
- **EU Regulation on Orphan Medicinal Products** in 1999

Pioneering orphan drugs through therapies for Lysosomal Storage Disorders (LSDs)

- **Very rare** diseases (<10,000 patients worldwide)
- Heterogeneous, progressive, debilitating diseases
- **Missing enzyme** causes health problems
- Genzyme produces missing enzyme by biotech techniques - treatments replace enzymes
- Introduced the first product approved to treat a LSD (1991)
- Marketed products for LSDs:
 - Fabrazyme® (algalsidase beta) for Fabry disease
 - Cerezyme® (imiglucerase for injection) for Type 1 Gaucher disease
 - Aldurazyme® (laronidase) for MPS-I
 - Myozyme® (alglucosidase alfa) for Pompe Disease



Our Mission, together with Stakeholders

- Optimizing **Patient Care** for unmet medical needs
 - Research
 - Diagnosis and/or appropriate screening
 - Clinical trials
 - Therapy development
 - Registry set-up
 - Delivery
- Helping to Build **Sustainable Healthcare Systems**

