The value and specificity of the rare disease business model

*The point of view from a dedicated orphan drug company*

Philippe Van Holle
International Chairman, Celgene

6th European Conference on Rare Diseases and Orphan Products
Brussels, May 24th 2012
Rare Diseases and Orphan Drugs are at the heart of what we do at Celgene

~90% of Celgene portfolio is composed of Orphan Drugs
Orphan Drugs: from cradle to .... adolescence

- The Orphan Drug Regulation (EC) 141/2000, together with national incentives, have contributed to the discovery and development of much needed drugs, led by a dynamic and highly innovative sector.

- However, Orphan Medicinal Product (OMP) companies, which have a unique model, typically face greater development and financial risks.

- Orphan Drug expenditures are expected to account for less than 5% of total European pharmaceutical expenditures by 2020, confirming both the affordability of Orphan Drugs and the sustainability of this new model for Health Care Systems.
Orphan Drugs: from cradle to .... adolescence

- The Orphan Drug Regulation (EC) 141/2000, together with national incentives, have contributed to the discovery and development of much needed drugs, led by a dynamic and highly innovative sector.

- However, Orphan Medicinal Product (OMP) companies, which have a unique model, typically face greater development and financial risks.

- Orphan Drug expenditures are expected to account for less than 5% of total European pharmaceutical expenditures by 2020, confirming both the affordability of Orphan Drugs and the sustainability of this new model for Health Care Systems.
Rare diseases have been ignored for a long time: prior to the introduction of the Orphan Drug Regulation, almost no products had been developed.

Drugs for rare diseases receiving marketing authorisation in Europe

Source: Assessment of the impact of OMPs on the European Economy and Society. OHE November 2010
This success is largely due to the unprecedented research efforts in Europe, triggered by the Orphan Regulation.

Annual Orphan drug R&D expenditure in the EU has more than tripled from €158m (2000) to €490 Million (2008).

European investment in orphan drug R&D 2000 and 2008 (€m)

Source: Assessment of the impact of OMPs on the European Economy and Society. OHE November 2010
Investment has primarily been driven by the period of exclusivity introduced by the Orphan Regulation.

Key features of the EU OMP legislation (ranked ‘Most important’ to ‘Third most important’)

Source: Assessment of the impact of OMPs on the European Economy and Society. OHE November 2010
Companies with a focus on rare diseases make a significant economic contribution in Europe

Number of people employed in orphan drug related activities in Europe, 2000 - 2008

Source: Assessment of the impact of OMPs on the European Economy and Society. OHE November 2010
Orphan Drugs: from cradle to .... adolescence

- The Orphan Drug Regulation (EC) 141/2000, together with national incentives, have contributed to the discovery and development of much needed drugs, led by a dynamic and highly innovative sector.

- However, Orphan Medicinal Product (OMP) companies, which have a unique model, typically face greater development and financial risks.

- Orphan Drug expenditures are expected to account for less than 5% of total European pharmaceutical expenditures by 2020, confirming both the affordability of Orphan Drugs and the sustainability of this new model for Health Care Systems.
OMP companies typically face greater development risk, with a small proportion of Orphan designated products receiving marketing authorisation.

Source: EMA (2011)
While the number of Orphan Designations increased over time, the proportion of successful Marketing Authorisations remained stable over time.

Source: EMA (2011)
As European Biotechnology companies, OMP companies also face greater financial risk.

Survival Index (cash reserve years) for European Biotechnology companies.

Source: Ernst & Young (2011)
Biotechnology investment is especially risky and investors require high returns on the few products that are successful to counter all the losses on companies and drugs that fail.

In a study of 180 biotechnology companies, less than half of the investors recouped the money they had invested.

In aggregate, the Biotechnology industry is still losing money each year.

Even with successful products, Biotech companies are failing to recoup all the investment on research and development.

Source: Ernest & Young (2009)
Orphan drugs are not exempted from competitive pressure, with several approved orphan drugs losing marketing exclusivity from 2011

Of 73 indications in which current orphan drugs have marketing exclusivity, 30 will have expired by end of 2016 and 23 of these will have no further patent protection and be subject to generic competition.

Cumulative orphan drug indications losing marketing exclusivity in Europe

Orphan Drugs: from cradle to ... adolescence

- The Orphan Drug Regulation (EC) 141/2000, together with national incentives, have contributed to the discovery and development of much needed drugs, led by a dynamic and highly innovative sector.

- However, Orphan Medicinal Product (OMP) companies, which have a unique model, typically face greater development and financial risks.

- Orphan Drug expenditures are expected to account for less than 5% of total European pharmaceutical expenditures by 2020, confirming both the affordability of Orphan Drugs and the sustainability of this new model for Health Care Systems.
In aggregate, the projected future cost of orphan drugs in Europe is likely to plateau over the next 10 years.

Orphan drug expenditure is expected to account for less than 5% of total European pharmaceutical expenditures between 2010 and 2020.

Projected future budget impact of orphan drugs in Europe as % of total European pharmaceutical expenditure

Orphan Drugs: from adolescence to ... maturity

- The development of orphan drugs remains highly dependent on positive incentives due to the high uncertainty and complexity inherent to orphan drug development, and to OMP companies’ unique economic model.

- While emerging interest of “Big Pharma” in the Orphan drug space has ensured successful commercialization of some Orphan drugs, it raises important questions...
Celgene remain committed to improve the lives of patients with rare diseases with development programmes in more than 40 rare diseases.

~90% of Celgene portfolio is composed of Orphan Drugs.
Working for Patients means...

Working with Patients!!!