Innovation needed in building a pathway from diagnosis to access

International conference on rare diseases
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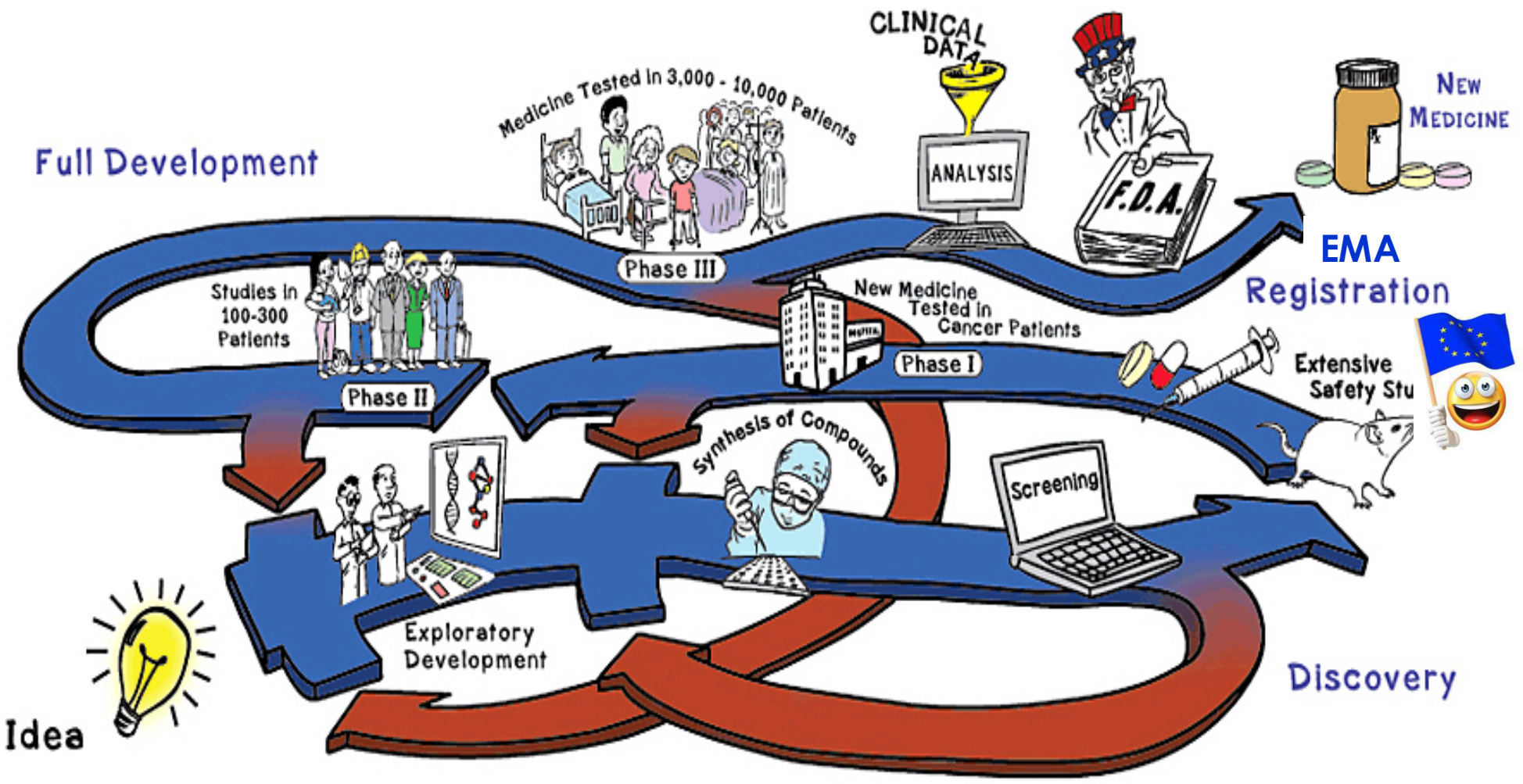
Developing medicines: a lengthy and complex undertaking



More complex in rare diseases

- Limited knowledge of diseases
- Conditions difficult to characterise
- Limited access to quality data
- Difficulty to power clinical trials
- Higher challenge to attract investment
- High risk profile for SMEs

The nature of the RD challenge to fix

- Almost all (>98%) of the people with rare diseases have one of the 390 most prevalent diseases (more than one in 100,000)
- Most (89.1%) of the rare diseases are very rare (less than 1 in 100,000)

Source Eurordis

- Any evaluation of the EU legislation on medicines for children and rare diseases should build on the current success of the existing legislation to increase and accelerate innovation

The importance of incentives in the development of medicines

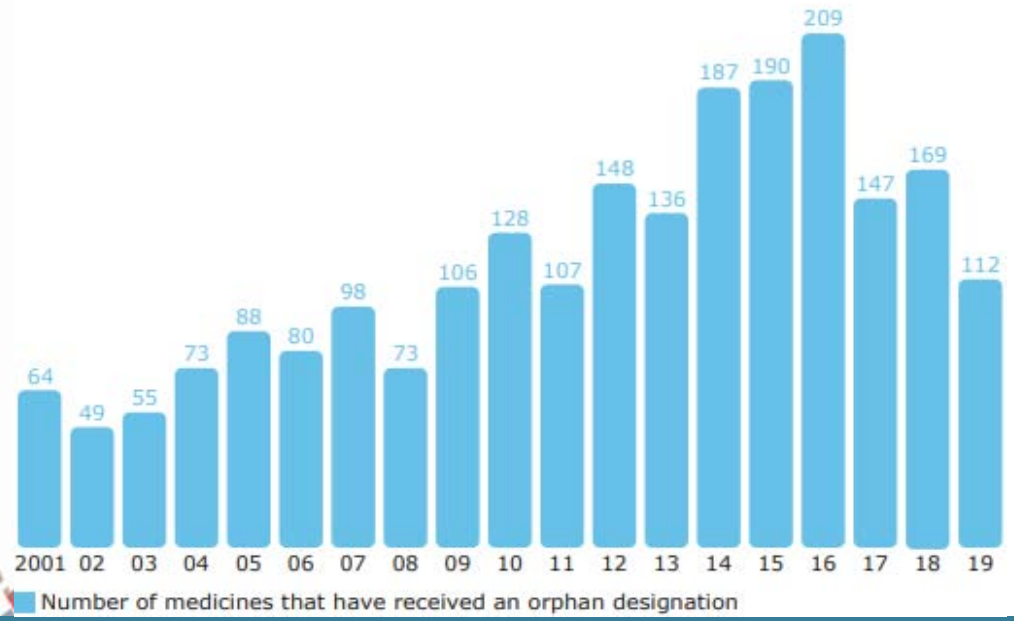
EuropaBio

over 2200 medicines with orphan designation

EMA's Committee for Orphan Medicines

The Committee for Orphan Medicinal Products (COMP) is in charge of reviewing applications for orphan designation.

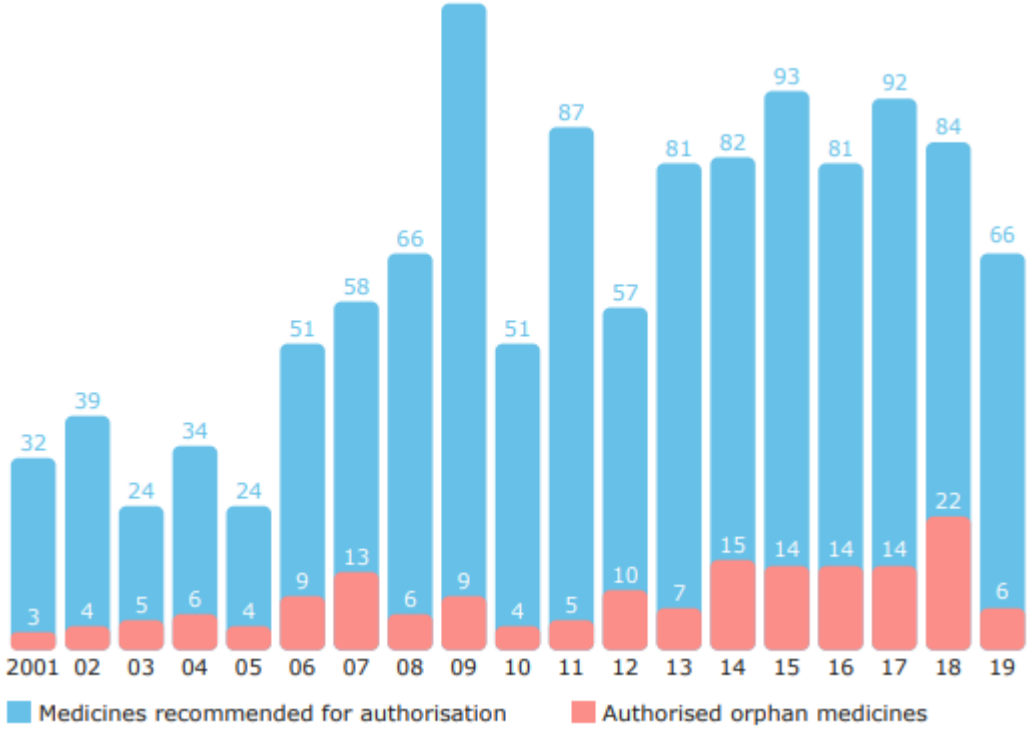
If a medicine makes it to the marketing authorisation stage, the COMP will assess it again to check whether the criteria are still met and the orphan designation can be maintained for the authorised medicine.



over 160 orphan medicines authorised in the EU

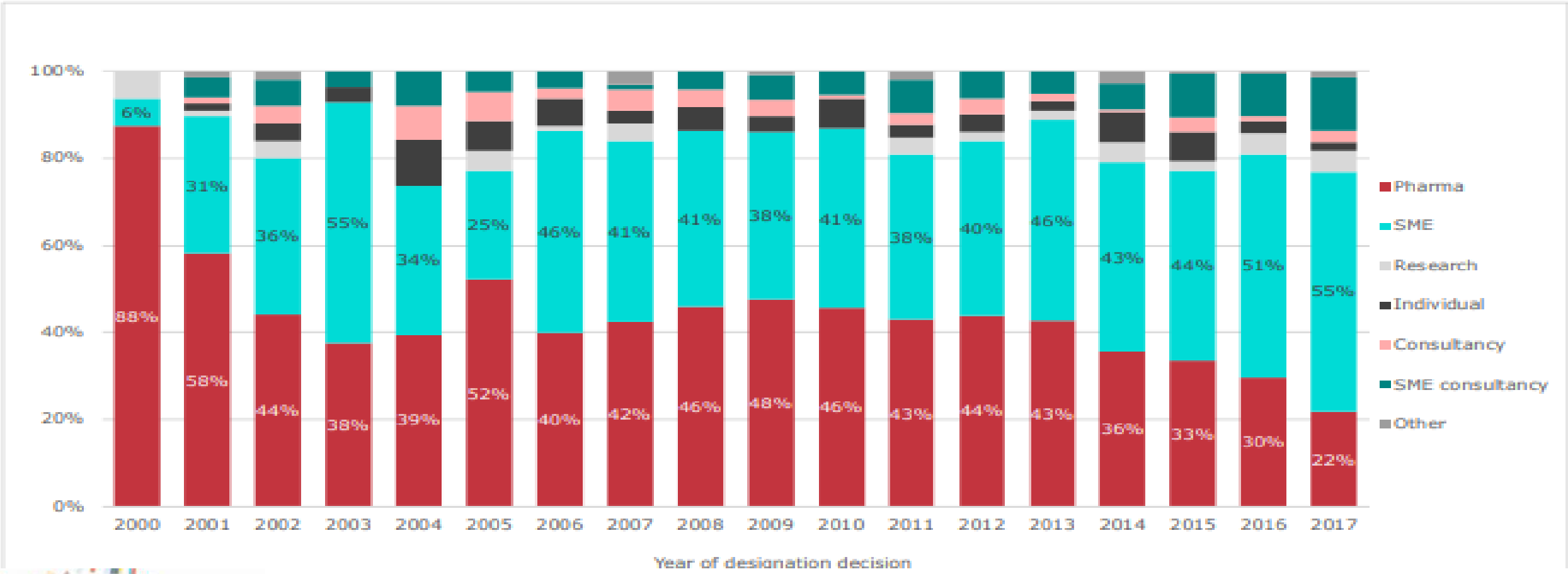
How orphan medicines reach patients

Once an orphan medicine is authorised by the European Commission, it can be marketed in all EU Member States. However, availability and reimbursment are subject to review by the relevant national authorities.



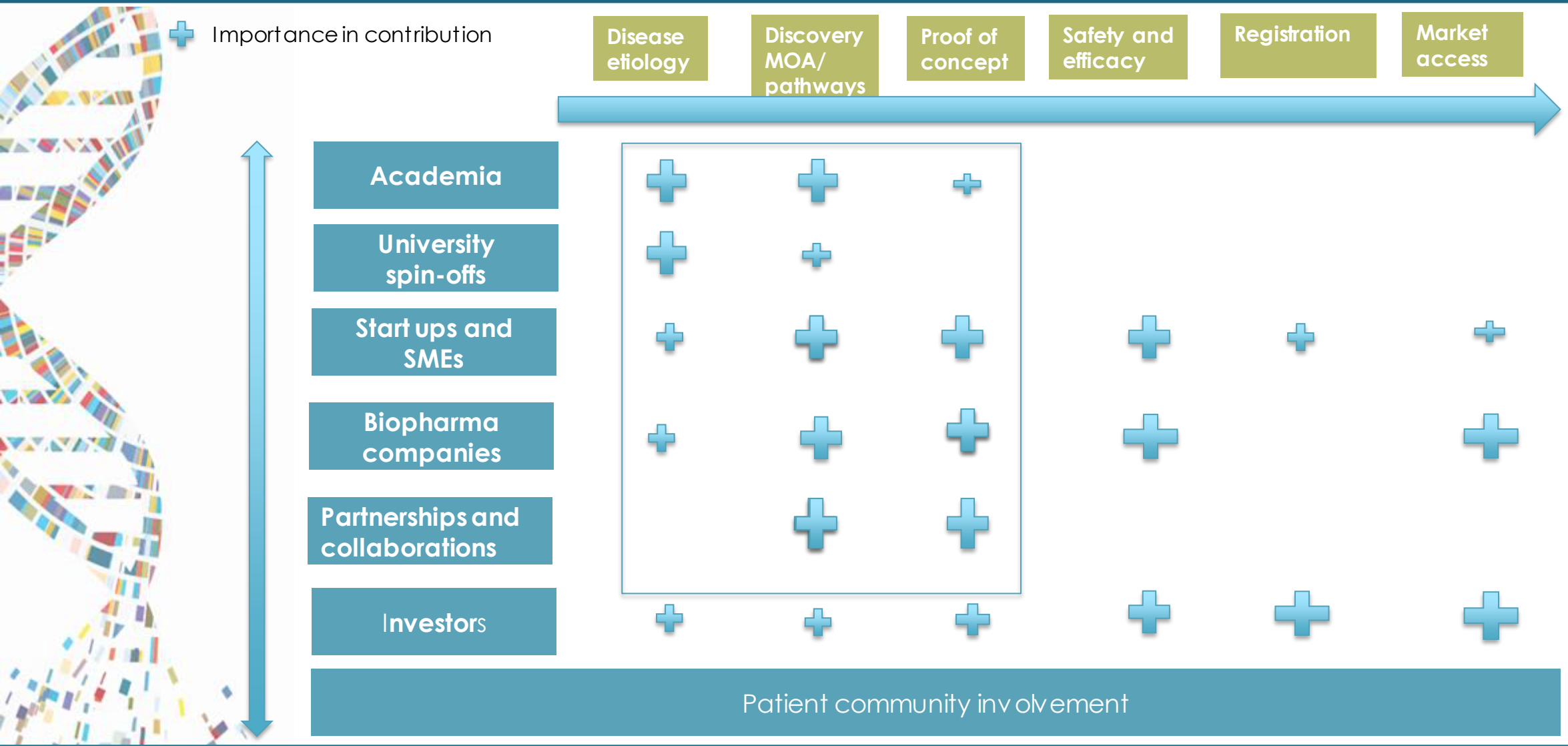
20 years of the OMP Regulation

Figure 28 Share of designations granted annually by type of sponsor



Source: European Commission study to support the evaluation of the EU Orphan Regulation, Final Report, July 2019

A European ecosystem that needs to be further nurtured



Working together to foster the Rare Diseases ecosystems

- **Strengthen the communities of care from Research to Patient Care**
  - **Enhance Academia- Industry collaboration**
  - **Increase Clinical trials knowledge and expertise**
  - **Make the best use of quality data, registries, RWE and power ERNs fully**
- **Strengthen the innovation engine for rare diseases**
  - **Create additional SME funding**
  - **Enhance R&D productivity**
  - **Implement innovative access solutions and streamline processes**

The right framework to build the innovation pipeline in rare diseases

- Incentives for all types of innovations are needed: breakthrough, incremental, repurposed
- Unmet medical needs should be defined broadly and encompass areas where patients still need better outcomes
- The patients are pivotal to define the unmet needs
- Leverage the revolution in biological sciences from molecular biology , computing and data processing by creating the right infrastructures in Europe
- Make sure European academia ,start ups and SMEs have access to these new technologies and are competitive on the world stage

Thank you

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