



How to ensure timely access to innovative medicines in Europe

The point of view of patients

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EURORDIS Rare Diseases Europe at a glance

755

member patient organisations



65 countries
(28 EU countries)

41 National Alliances of RD Patients Organisations

58 European Federations of specific rare diseases

 Founded in
1997



40+

Staff members,
with offices in
Paris, Brussels,
Barcelona

Outreach to over

1800

patient groups

  Over

440

Volunteers

+80
patient
advocates

+250
moderators

What is a rare disease?

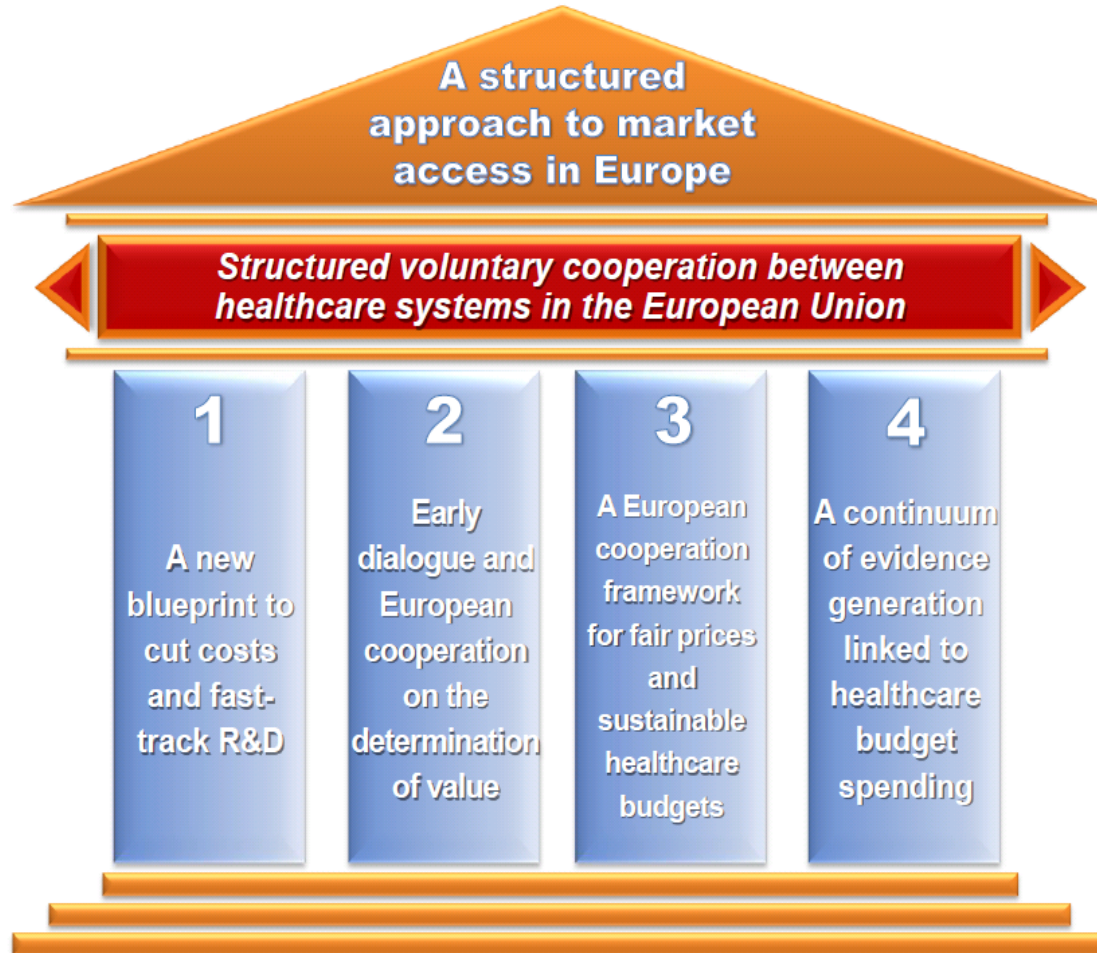
A rare disease is defined in Europe as affecting
less than 1 in 2,000 citizens

- Rare diseases are **chronic, progressive, degenerative, disabling** and frequently **life-threatening**
- Over **6,000** distinct rare diseases, many are of **genetic** origin
- **95%** of diseases **do not** have an approved therapy
- **30 million** people living with a rare disease in Europe
- **6%** of the population affected in the course of their lives
- Many **children** are affected by rare diseases. Onset occurs in childhood for 50% of rare diseases.

The challenges

- Patients and experts are few, geographically **scattered** and often **isolated**
- Patients **are undiagnosed, misdiagnosed or wait years for a diagnosis**
- Reliable **information is scarce**
- **Resources** are limited
- Lack of **treatments and challenges** to access adequate care
- **Fragmented research**, data and information
- High social impact and **marginalisation** within society at large and within healthcare systems designed for common diseases
- **Heavy psychosocial burden**: societal support is essential to patients and families to enable them to cope, be resilient, care for others

The European added value of cooperation in rare diseases





THANK YOU

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