ERDERA

European Rare Diseases
Research Alliance

Unlocking Access: Orphan Drugs in the EU

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I have no real or apparent relevant financial relationships to disclose

DOI

www.ema.europa.eu/docs/en_GB/document_library/contacts/athanasioud_DI.pdf

What Are Orphan Drugs?

- - Medicines developed for rare diseases
- - 'Orphan' because few patients = less financial incentive
- - 1 in 17 people will be affected by a rare disease in their lifetime

Why Are They Important?

- MANY RARE DISEASES HAVE NO TREATMENT

- ORPHAN DRUGS OFFER HOPE AND LIFE-SAVING OPTIONS

- ESPECIALLY IMPORTANT FOR YOUNG PEOPLE WITH LIFELONG CONDITIONS

Challenges to Access in the EU

- ✗ High cost (some drugs > €200,000/year)
- ✗ Long approval processes
- ✗ Not all EU countries reimburse them
- ✗ Inequality between Western and Eastern Europe

What Helps Improve Access?

- ✅ EU Orphan Drug Regulation
- ✅ EMA (European Medicines Agency) fast-tracks approval
- ✅ National health systems can provide funding
- ✅ Patient organizations raise awareness

Real Stories Matter

- Patients' voices help drive policy!

- Stories show real impact of not having access

- Youth can collect and share these stories

What Can YOU Do as Young Advocates?

- Learn about rare diseases in your country
- Talk about access issues on social media
- Support awareness days
- Join youth advocacy campaigns

Examples of Youth in Action

- Youth Rare Disease Network (Europe-wide)

- Teen-led webinars on orphan drugs

- Campaigns that helped get funding for treatments

Final Thoughts

"Access is not a privilege—it's a right."

Your voice can help make orphan drugs available to everyone.

Stand up. Speak out. Be the change.

Resources & Support

- EURORDIS (Rare Diseases Europe)

- National rare disease alliances

- Your local patient organization

THANK YOU!

ATHANASIOU DIMITRIOS, BA, MBA, World Duchenne Organization / UPPMD Board Member, EMA PDCO Member

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Thank you!

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