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Improving Access to Orphan Drugs Through International Health Policy

Francois Houÿez

EURORDIS – Rare Diseases Europe, France

The development of orphan drugs has transformed the treatment landscape for many rare diseases, offering new therapeutic possibilities for conditions that were once considered untreatable. However, scientific innovation alone is insufficient to improve patient outcomes unless it is supported by equitable healthcare policies that ensure timely, affordable, and sustainable access to these therapies. Despite significant advances in biotechnology, gene therapy, and precision medicine, patients with rare diseases continue to face substantial barriers to treatment due to differences in regulatory pathways, reimbursement policies, healthcare infrastructure, and national funding mechanisms.

This presentation examines the current landscape of orphan drug policy and explores how international collaboration can improve access to innovative therapies for individuals living with rare diseases. It reviews the evolution of orphan drug legislation across Europe and other regions, highlighting regulatory incentives that have encouraged pharmaceutical innovation while identifying ongoing challenges related to pricing, market sustainability, and equitable distribution.Finally, the presentation explores future policy directions aimed at strengthening global access to orphan medicines through regulatory harmonization, sustainable funding models, innovative reimbursement strategies, and expanded international cooperation.

Biography

François Houÿez is Scientific Director at EURORDIS – Rare Diseases Europe and a recognized expert in orphan drug policy and rare disease advocacy. He has worked extensively with European regulatory authorities, patient organizations, and healthcare policymakers to improve access to innovative therapies. His expertise spans health policy, regulatory science, and international collaboration aimed at advancing patient-centered care for rare diseases.