Unmet medical needs in rare disease: A case study from Amyloidosis

Hosted by

MEP **Susana Solis Perez**

renew europe.

04 July 2023
8:15 – 9:30 CET
MEP Salon
European Parliament
60 rue Wiertz
1047 Brussels

Panel conversation followed by a breakfast

Join us for an empowering discussion dedicated to raising awareness of rare diseases and the impact they have on patients and European health system and look towards the future of the European life sciences sector.

During the event, policymakers and experts will draw upon the example of amyloidosis – a group of underdiagnosed and under recognised rare diseases impacting thousands of patients.

08:15 WELCOME AND HOUSEKEEPING / **MEP Susana Solis Perez** (Renew, Spain)

08:20 INTRODUCTORY REMARKS

08:25 PANEL DISCUSSION

Alexander Natz
Director General, EUCOPE

Jean-Christophe Fidalgo
President, World Amyloidosis Alliance

Simone Boselli
Public Affairs Director, EURORDIS

09:10 Q&A

09:25 CLOSING REMARKS / **MEP Susana Solis Perez** (Renew, Spain)

Event description

This panel conversation aims to raise awareness on rare diseases and on their impact on patients and European health systems. It is a critical juncture for rare disease policy. Indeed, the EU pharmaceutical legislation on rare diseases[1] is to be revised and the Spanish Presidency is to set rare diseases as a priority. This political momentum offers a window of opportunity to recognize and meet the needs of over 36 million people affected by rare diseases in the EU[2].

Amyloidosis is one of the thousands of rare diseases affecting EU citizens. It is a complex and multifaceted disease which manifests itself in six different forms; the common characteristic being that they are all caused by an abnormal build-up of proteins, which deposit as amyloid in tissues and affect organs negatively, generally the heart, kidneys, peripheral nervous system, liver and digestive system.[3,4] Amyloidosis is detected in 6 to 10 million inhabitants per year in Western countries, but diagnostic delay is a major issue and while there are care options to help manage symptoms, there is no cure[5,6].

In light of the great unmet medical need of people living with rare diseases and with amyloidosis in particular, it is crucial that the EU encourages the development of treatments for rare diseases by incentivizing innovation. This is a strategic stake for the revision of the EU legislation on rare diseases as part of the 'Pharmaceutical Package'. The EU must seize this opportunity to improve the health and quality of life of the 36 million citizens living with rare diseases and to create a leading life science sector on a global scale.

1. European Commission 2. Expert Group on Public Health 3. Amyloidosis Alliance
4. John Hopkins Medicine 5. World Amyloidosis Day 6. National Health Service

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