

IQWIG AUTUMN SYMPOSIUM 2023

The challenge of Rare Diseases

Programme on 24./25.11.2023

Chair: Holger Wormer

Friday, 24.11.2023 | Day 1

- 13:30 – 13:45 Welcome and introduction**
Holger Wormer (moderator), Thomas Kaiser (IQWiG, Cologne)
- 13:45 – 14:45 Living with a rare disease: searching, fighting, hoping**
Mirjam Mann, Christine Mundlos, Michael Scholz (ACHSE e. V., Berlin)
- 14:45 – 15:30 From the perspective of the pharmaceutical industry: Why is clinical research so difficult here?**
Christiane Denzel (Alexion Pharma Germany GmbH, München)
- 15:30 – 16:00 Coffee break**
Individual discussions
- 16:00 – 16:45 Diagnostics of rare diseases with AI support**
Martin C. Hirsch (Philipps University, Marburg)
- 16:45 – 17:30 A health economic view on the cost structure in the drug market, including orphan drugs**
Julian Witte (Vandage GmbH, Bielefeld)
- 17:30 – 18:15 Moderated discussion: Are the currently high expenditures for orphan drugs justifiable and affordable in the long term?**
Martin J. Hug (Hospital pharmacy, University Hospital, Freiburg),
Wolf-Dieter Ludwig (Drug Commission of the German Physicians, Berlin)
- from 18:15 Get-together**
Dinner – individual discussions

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Saturday, 25.11.2023 | Day 2

- 9:00 – 9:45** **Drug approval in the field of rare diseases – the regulatory perspective**
Martina Schüßler-Lenz (Paul-Ehrlich-Institute, Langen)
- 9:45 – 10:30** **Methodological approaches to generate robust evidence in rare diseases**
Ralf-Dieter Hilgers (RWTH Aachen)
- 10:30 – 11:00** **Coffee break**
Individual discussions
- 11:00 – 11:45** **Patient registries and natural-history studies to support evidence generation for rare diseases**
Thomas Klopstock (Ludwig-Maximilians-University, München)
- 11:45 – 12:30** **Patient registries on the way to (randomized) controlled trials**
Lutz Nährlich (University Hospital, Gießen/Marburg)
- 12:30– 12:45** **Final discussion**
Holger Wormer (moderator), Thomas Kaiser (IQWiG, Cologne)
- from 12:45** **Farewell**
Lunch