Let’s make a pact to ensure patients’ sustainable access to rare disease therapies

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Celgene, Novartis, Pfizer, Takeda

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RATIONALE & OBJECTIVES

PANEL discussion: outlining interests by the key constituencies

10.15 – 10.45 Coffee break

09.55 – 10.15

There is a new treatment approved, when can I get it? The example of SMA

09.45 – 9.55

09.15 – 09.30

09.00 – 09.15

Sheela Upadhyaya

Moderator:

Sheela Upadhyaya, Global Healthcare Portfolio Manager, ROBECO

Patient perspective: Dimitrios Athanasiou, Director General, EFPIA

Panelists:

José Manuel Mencía de Lemus, Secretary of State, Ministry of Health of Romania

Tiberius-Marius Brăcanu, Secretary of State, Ministry of Health of Romania

Yann Le Cam, Secretary General, European Confederation of Private Health Insurance (COPAM)

Diego Ardigò, Speaker:

University Hospital Aachen, Institute Director, Universitätsklinikum Aachen

Mark Turner, Epidemiologist, Academic Medical Center (AMC)

Hanneke van der Lee, Consultant, HTAi

Karen Facey, Rare Disease Policy Manager, Newcastle University; John Walton Muscular Dystrophy Support Group; Head of Market Access (TTR), Alkta UK Support Group

Sandra Nestler-Parr, Project Advisor, MoCA

11.30 – 12.00

Rational and Objective

DAY 1
WEDNESDAY 13 FEBRUARY 2019
09.00 to 17.00

Morning session until 13:00 video stream and open to everyone

09.00 – 10.30 OPENING PLenary Session

Why is it a priority?

09.00 – 09.15

Introduction

Sandra Nestler-Parr, Co-Chair, Rare Disease Special Interest Group, ISPOR; Trustee, Alkta UK Support Group; Head of Market Access (TTR), Alkta UK Support Group

09.15 – 09.30 Welcome and opening remarks Yara Le Can, Chief Executive Officer, EURORDIS-Rare Diseases Europe

09.30 – 09.45 Keynote speech Tiberius-Marius Brăcanu, Secretary of State, Ministry of Health of Romania

09.45 – 10.00 There is a rare diseases treatment approved, when can I get it? The example of SMA

10.00 – 10.15periences of rare diseases, how do we address the needs of end-users through implementation tests to determine the value of a treatment. Patients’ views to traditional clinical development programmes may not be

10.15 – 11.00 Breakout Session 1: A new blueprint to decrease unnecessary hardship and improve the situation for patients

11.00 – 11.45 Breakout Session 2: Implementing Multi-Stakeholder Early Dialogues to Optimize Determination of Value

11.45 – 12.15 Breakout Session 3: A new approach to speed up efficiently and fast-track R&D

12.15 – 13.00 Breakout Session 4: Improving Multi-Stakeholder Early Dialogues to Optimize Determination of Value

13.00 – 13.30 Farewell lunch

13.30 – 14.30 Feedback session (plenary)

14.30 – 15.15 Breakout Session 3: A new approach to speed up efficiently and fast-track R&D

15.15 – 16.00 Breakout Session 4: Improving Multi-Stakeholder Early Dialogues to Optimize Determination of Value

16.00 – 16.30 Feedback session (plenary)

16.30 – 17.30 Coffee break

THURSDAY 14 FEBRUARY 2019
09.00 to 17.30

10.00 – 10.30 OPENING PLenary Session

Why is it a priority?

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16.00 – 16.30 Breakout Session 4: Improving Multi-Stakeholder Early Dialogues to Optimize Determination of Value

16.30 – 17.15 Wrap-up and closing remarks Yara Le Can, Chief Executive Officer, EURORDIS-Rare Diseases Europe