



# Support for development of orphan medicines

**Incentives, scientific advice, and funding schemes**

30 November 2020, 09:00 – 13:00



## Main aims of the workshop

---

- To stimulate and facilitate development of innovative drugs and in particular development of orphan medicines in neglected disease areas;
- To encourage early interactions with the regulators by highlighting the impact of pre-marketing support for medicine development in rare diseases;
- To highlight existing incentives such as protocol assistance for development of orphan medicines and how to efficiently use them;
- To learn from the stakeholders about the difficulties and perceived value of their interactions with regulators.

## Directions for joining the workshop

---

The workshop will be broadcasted live. You can follow the broadcast available on the 'Live broadcast' section on the event page on the day of the event. Audience participation will be encouraged by using an interactive polling platform, through which participants can share their views on a range of issues discussed during the workshop. A video recording will be available after the event.

## Additional information


---

Year 2020 marks the 20th anniversary of the introduction of the Orphan Regulation in the European Union and which coincides also with its evaluation by the European Commission. Despite objective successes of the Orphan Regulation, many disease areas have still not attracted sufficient attention and need more dedicated research.

This virtual event aims to encourage early and efficient interactions with the regulators by highlighting pre-marketing support for medicine development in rare diseases. Existing tools such as orphan designation, protocol assistance and PRIME scheme will be explained in the context of early product development strategy. Furthermore, the COMP will illustrate key elements essential for a successful orphan designation application.

Stakeholders will have an opportunity to express their views in relation to early medicine development and regulatory interactions and to participate in related Q&A sessions.

The workshop is targeted at small to medium enterprise medicine developers, academia, patients, healthcare professionals and ERNs who are often at the forefront of medicine development in rare and neglected diseases.





# Support for development of orphan medicines

Incentives, scientific advice, and funding schemes

*Chaired by Violeta Stoyanova-Beninska*

## Introduction

---

<b>08:30 – 09:00</b>	<b>Registration</b>	<b>30'</b>
<b>09:00 – 09:15</b>	<b>Welcome and introduction by EMA Management and Chair</b> <i>Michael Berntgen</i> <i>Violeta Stoyanova-Beninska</i>	<b>15'</b>
<b>09:15 – 09:20</b>	<b>Objectives of the workshop</b> <i>Violeta Stoyanova-Beninska</i>	<b>5'</b>

## Orphan development support

---

<b>09:20 – 09:45</b>	<b>Orphan designation and Orphan Medicines in EU</b> <i>Frauke Naumann-Winter</i> <i>Darius Matusevicius</i>	<b>25'</b>
<b>09:45 – 10:00</b>	<b>EMA resources for academics and SMEs</b> <i>Helene Casaert</i>	<b>15'</b>
<b>10:00 – 10:15</b>	<b>IRDIRC Orphan Drug Development Guidebook</b> <i>Virginie Hivert</i>	<b>15'</b>
<b>10:15 – 10:30</b>	<b>Questions and discussion</b> <i>Elisabeth Penninga</i>	<b>15'</b>

## Pre-marketing incentives for development in rare diseases

---

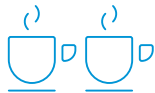
<b>10:30 – 10:50</b>	<b>Protocol Assistance/PRIME/parallel consultation</b> <i>Armando Magrelli</i>	<b>20'</b>
----------------------	---	------------





**10:50 – 11:05** **Current funding schemes** **15'**  
*Daria Julkowska*

**11:05 – 11:20** **Questions and answers** **15'**  
*Kristina Larsson*



**Coffee break**

## Stakeholders' perspective on the value of orphan designation

---

**11:45 – 12:00** **Academic perspective** **15'**  
*Juan Antonio Bueren (CIBER)*

**12:00 – 12:15** **SME perspective** **15'**  
*Andrea Braun-Scherhag (Ultragenyx, EUCOPE)*

**12:15 – 12:30** **Patients perspective** **15'**  
*Nick Sireau (AKU)*

**12:30 – 12:50** **Questions and answers** **20'**  
*Maria Sheean*

**12:50 – 13:00** **Wrap up** **10'**  
*Michael Berntgen*  
*Violeta Stoyanova-Beninska*



**Lunch break**

## IRIS Clinic (optional for participants)

---

**14:00 – 14:15** **Introduction to IRIS** **15'**  
*Paolo Tomasi*

**14:15 – 15:15** **Questions and answers** **60'**  
*Paolo Tomasi*  
*Monica Gomar*

