



# Pediatric Patients Expert Group

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January 24<sup>th</sup>, 2025

Online Meeting

<https://invents-he.eu/>



INVENTS has received funding from the European Union's Horizon Europe Research and Innovation program under grant agreement 101136365.



Funded by the Swiss State Secretariat for Education, Research and Innovation (SERI).



Funded by the UKRI Innovative UK under their Horizon Europe Guarantee scheme.



# Let's start by introducing ourselves



**Yanis Mimouni**  
Inserm, EJPRD  
France



**Donato Bonifazi**  
Teddy Network  
Italy



**Viviana Giannuzzi**  
Teddy Network  
Italy



**Francesco Lapenna**  
Teddy Network  
Italy



**Vincent Damotte**  
Inserm  
France



**Stéphanie Gentile**  
Inserm, APHM  
France



**Enora Leroux**  
Inserm, APHP  
France



**Vincent Montero**  
Inserm, OrphanDev  
France





# Discussion

Let's use MIRO to share your thoughts



# The INVENTS project

Clinicians are facing challenges in designing clinical trials for rare diseases

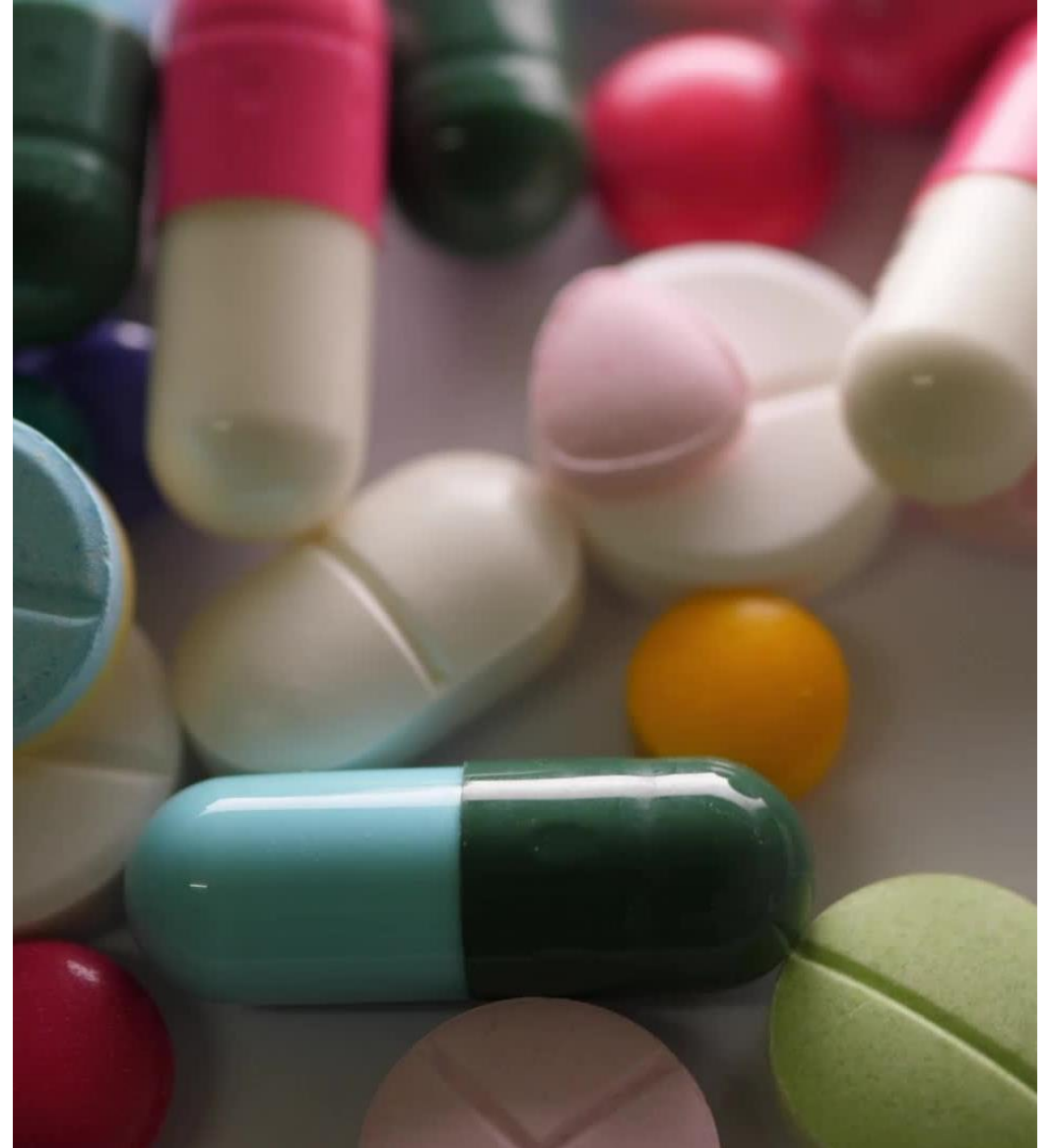
The INVENTS project aims at:

- Creating new methods for clinical trials design in rare diseases
- Making european and local regulatory agencies used those methods, and by taking into account patient experience



# Drug approval process

Did you understand the process from the discovery of a new drug to its approval for use by patients?

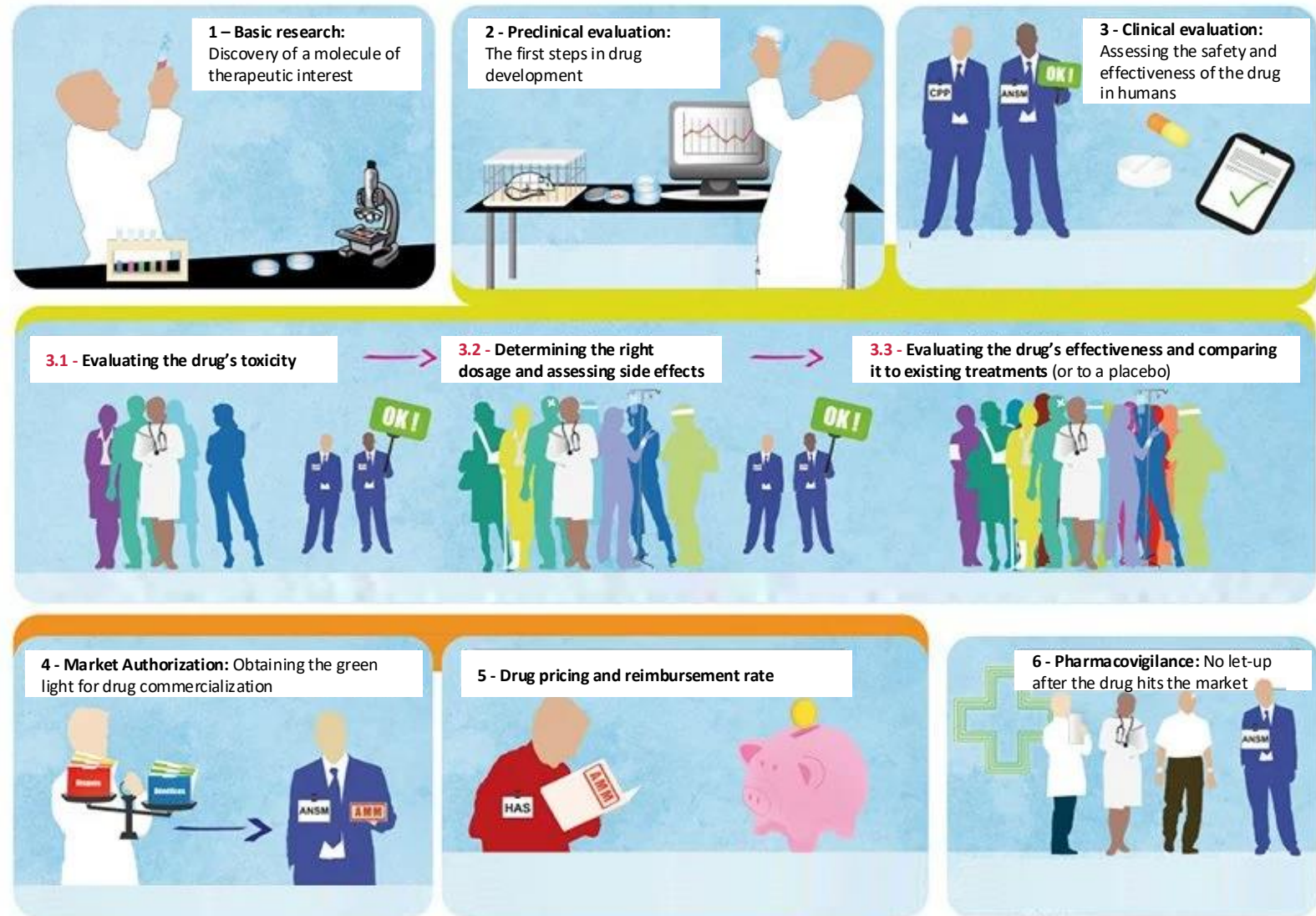


# Discussion

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# Drug approval process



Adapted from <https://www.inserm.fr/dossier/medicament-developpement/>



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# Patient experience

1. Did you understand why it is important for patients' opinions to be given even more consideration in the approval/rejection of new medications?
2. Did you remember the example given where patients were able to influence a treatment approach? Do you have an example you've heard of, or experienced, where authorizations were not granted or extended, even though patients felt they were benefiting?



# Discussion

Let's use MIRO to share your thoughts



# Patient experience

“Patient experience” means how a patient feels and what they go through when they are being cared for.

This includes everything that happens, like the care they get, talking to doctors, and even how they feel when they take medicine.





# Your opinion matters!



Would you like to give your opinion to help know what kind of new medication should be developed? To give your opinion on how this research should be conducted? This could also include the authorization process





# Interview Guide

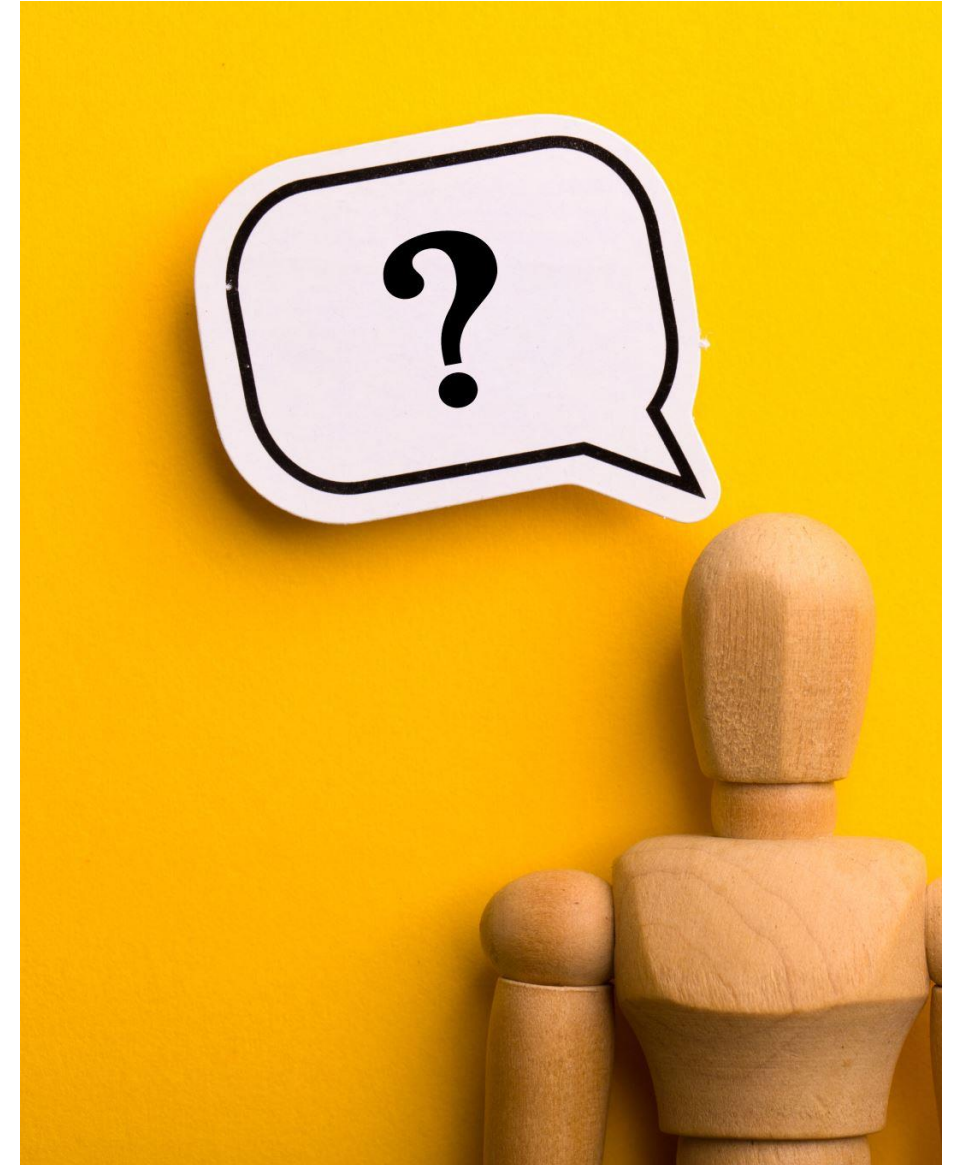


What do you think about the idea of involving ill people more in all these decisions? Would you like to be asked your opinion before they decide to develop a treatment or authorize a medication?



# Interview Guide

Do you have an example to share about something you live with or have experienced that could be improved and that people aren't aware of enough?





# Interview Guide

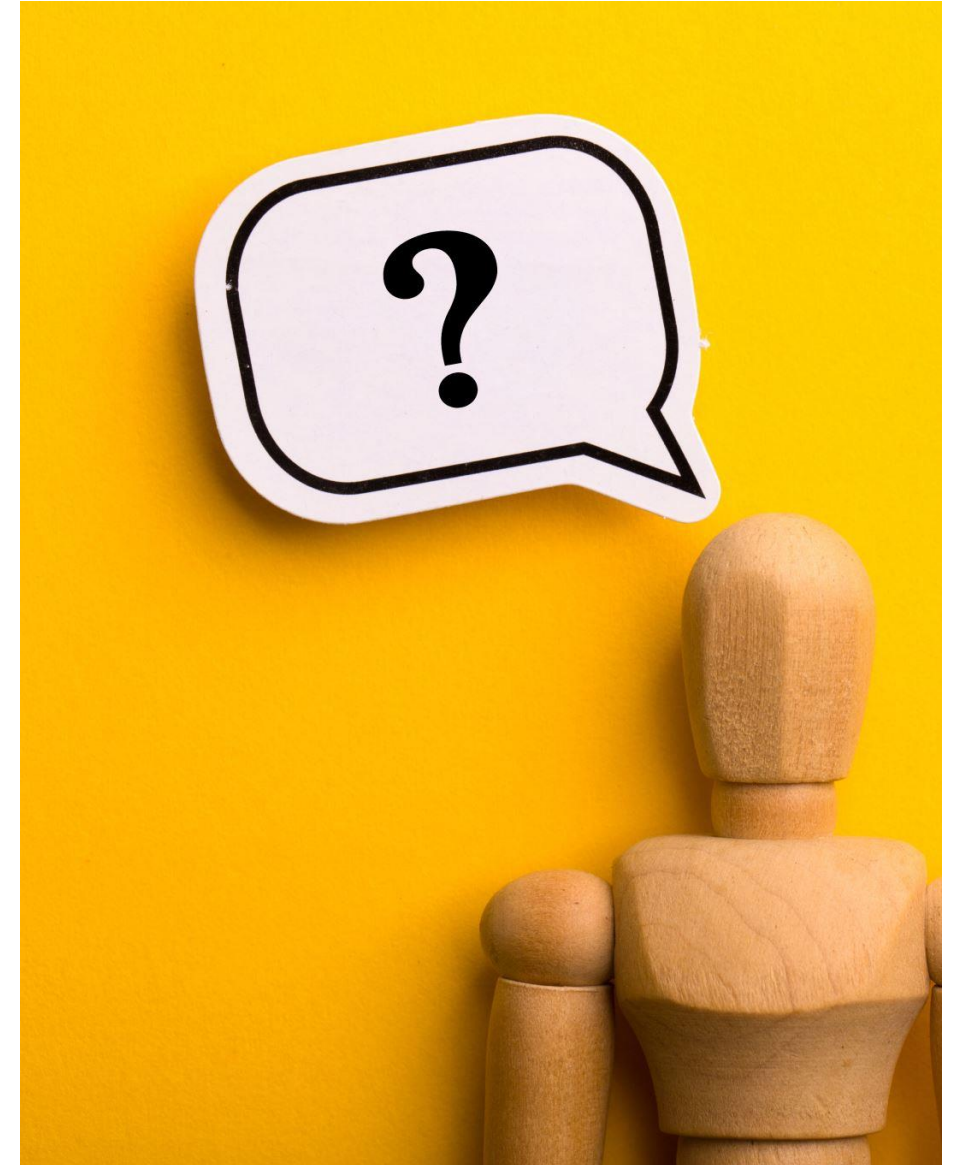


If you can't do it directly, who could speak on your behalf when important decisions need to be made? A doctor, a parent, or someone your age who's going through the same thing?



# Interview Guide

What could be developed so that you could give your opinion? (an app, a survey, or talking directly with a doctor, through your parents?)



# Specific objectives

- To give an overview of the rules and ethical recommendations to help develop and use new methods.
- To help sick children and their representatives take part in creating and evaluating new methods and make them easier.
- To create tools that measure what sick children experience, so the people making decisions can choose the right medicines.





Thank you for your participation

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