



Rare Diseases 360°

Collaborative Strategies to leave no-one behind

9th European Conference on Rare Diseases & Orphan Products

10-12 May 2018 Vienna

**Global access to
timely and accurate **DIAGNOSIS**
and
optimised **CARE****

A Patient perspective

Olivia Romero Lux



WFH

WORLD FEDERATION OF HEMOPHILIA
FÉDÉRATION MONDIALE DE L'HÉMOFILIE
FEDERACIÓN MUNDIAL DE HEMOFILIA

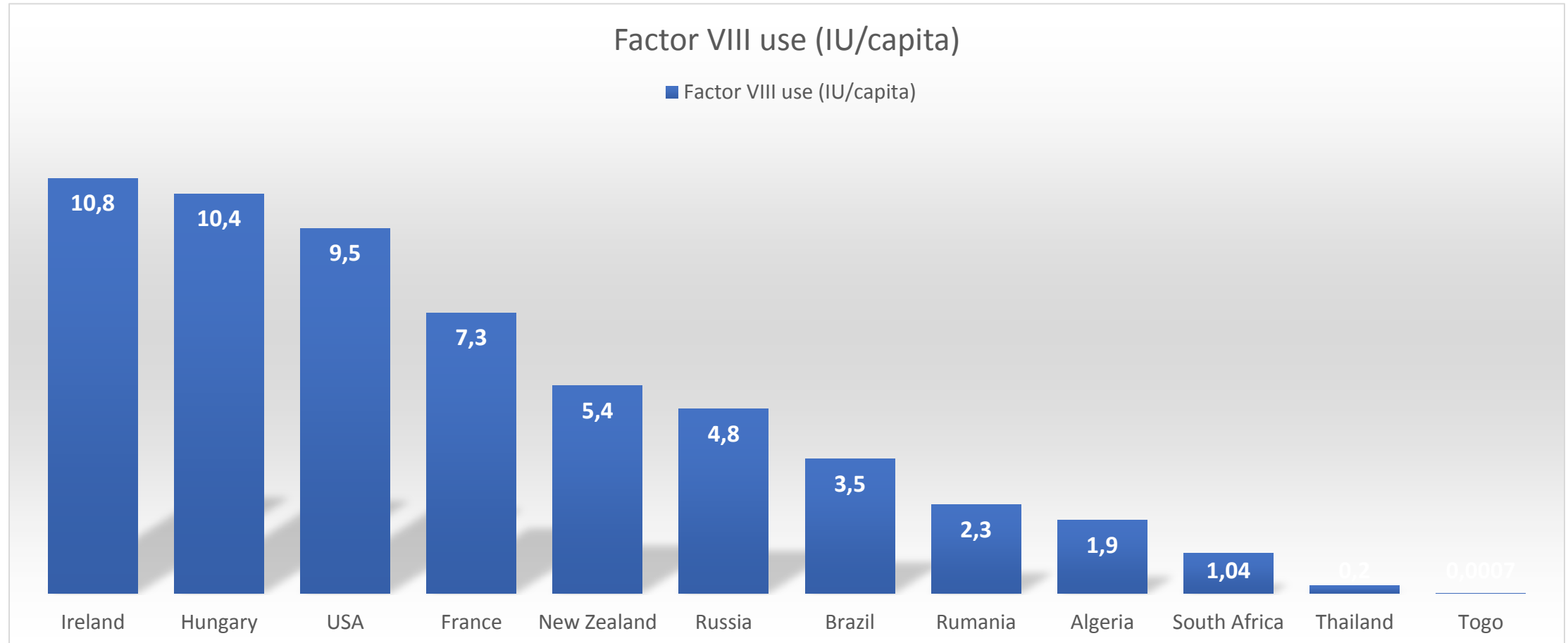
DISCLOSURE

Conflict	Disclosure - if conflict of interest exists
Research Support	no
Director, Officer, Employee	no
Shareholder	no
Honoraria	no
Advisory Committee	BioMarin and Roche
Consultant	No

What if I told you that...

There is a rare disorder where there have been
SAFE & EFFICACIOUS TREATMENTS
for over **20 years** now and
75% of the patients around the world
who have been diagnosed have still
NO ACCESS to them?

How do we assess level of CARE?



What if I told you that...

The **prevalence for haemophilia A** is **1 birth out of 10,000**.

**Diagnosed people with haemophilia
around the world in 2016:**

200,000

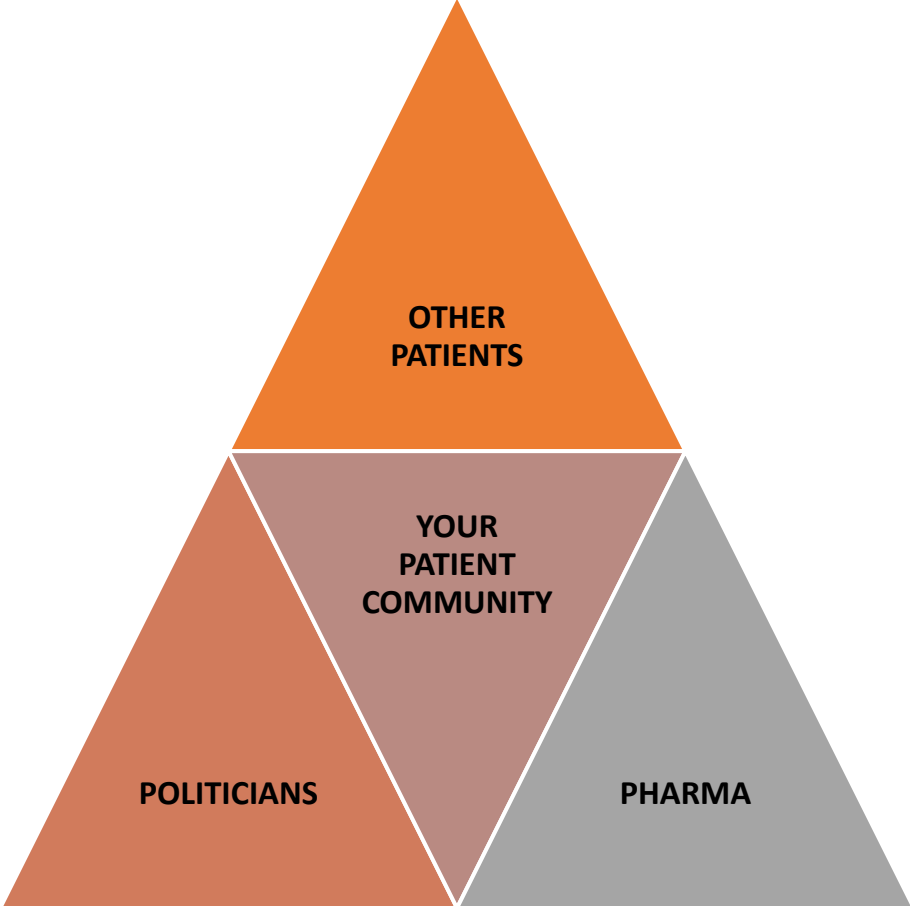
How do we assess level of **DIAGNOSIS**?

India: 1.4 billion inhabitants
18,000 people diagnosed
140,000 potential patients

UNDIAGNOSED people with haemophilia around the world in
2016:

400,000 or 70%

What's next for the PATIENT community...



What's next for OUR community...

- Because **NEW PRODUCTS** are being developed and will be granted market authorization
- Because **GENE THERAPY** is already way underway
- Because **NEW PATIENTS** need to be diagnosed
- Because diagnosed need to **ACCESS TO TREATMENTS**
- Because patients will always need **TO BE EMPOWERED**

COLLABORATION

THANK YOU!