

# Outcomes Measures in Haemophilia – Roche`s View

*Maher Aassi, MD  
Medical Director, Hemophilia, Roche*

EHC Roundtable  
Monday 28 November 2016  
Brussels, Belgium

## Three companies working as one in advancing haemophilia care



- Genentech, member of the Roche group, transformed the care and management of hemophilia A in 1984 by becoming the first company to clone recombinant factor VIII.
- Together, **Roche, Genentech, and Chugai** collaborate to advance innovative science around the globe, with a shared commitment to doing now what patients need next, including researching treatment for people with hemophilia A.



***‘Health is a state of complete physical,  
mental and social well-being and not merely  
the absence of disease infirmity’***

WHO Constitution

June 1946



**A new healthcare paradigm has emerged**



*Patient-centered outcomes*

*Shared decision making*

*Patients as partners*

*Value-based*

*Outcomes important to patients*

*Direct patient voice*

*Patient-focused drug development*

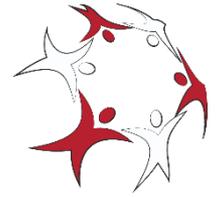
# Why Roche focuses on patient relevant outcomes



In haemophilia research, most endpoints are driven by the people with haemophilia, supplementing the clinically collected data.



## Roche recognises the critical nature of the patient voice in our research



- In contrast with some other diseases, people with haemophilia are the experts in the self-management of their disease
- As a result, they may seldom see specialist haemophilia healthcare professionals
- Thus, it is important that outcomes for haemophilia are collected directly from the patients
- We cannot assess the value of a treatment for haemophilia without considering key patient reported outcomes
- PRO`s are key endpoints, and measured routinely, in all of our pivotal trials:
  - Bleed events and treatments
  - HRQoL
  - Health status
  - Novel, patient focused, endpoints

## There is a scarcity of real-world evidence around patient reported outcomes in haemophilia



- Real world data collected directly from people with haemophilia are considered of great importance for the emicizumab clinical development program:
  - provide rich contextual information on current standard of care and patient burden of disease
  - enable intra-patient comparison for those who subsequently participate in a pivotal emicizumab Phase 3 study
- A non-interventional study was designed, utilizing an electronic PRO tool, to prospectively collect detailed, high quality, patient reported, real world data:
  - medication use and treatment regimen
  - Bleed onset, location and symptoms
  - HRQOL and health status (Haem-A-Qol; Haemo-QoL-SF; EQ-5D-5L)
  - missed work/school
- 260 patients are recruited in 3 cohorts: adults and adolescents with and without inhibitors and children with inhibitors



## Use of technology to improve the experience of people with haemophilia

- Technology can be leveraged to deliver care and information to patients and providers that can be more convenient, more cost effective and personalized
- It can also be used to track patient relevant data, associated with bleeds and treatment, including physical activity, and build high quality, research-ready, data.
- Digital health technology includes:

### Hardware

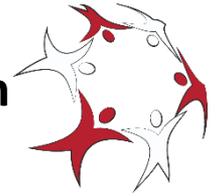
Cell phones  
Wearables  
Computers  
Cameras  
Robots  
Sensors  
Medical devices  
Monitoring equipment

### Software

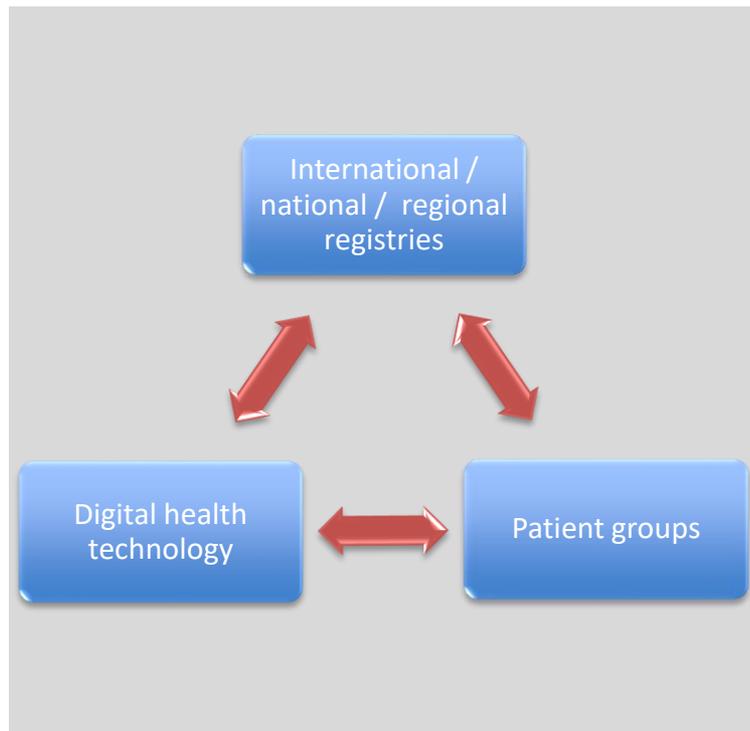
Mobile apps  
Text messaging  
Electronic health records  
Video conferencing  
Computer programmes  
Social media  
Artificial intelligence



# Intergrating the patient voice into routine data collection, through collaboration



Can we utilize new technologies and tools to seamlessly incorporate patient reported outcomes into national and regional registries, enabling more informative, patient centered, research?



Advantages of building PRO`s into central registries:

- Minimise additional burden to physicians and patients from ad hoc studies
- Impartiality – data are ‘owned’ by the community / country, not by pharma
- Invest in data – use resource to drive up quality and completeness
- Help establish a sustainable long-term model for data collection
- Patient groups can play a central role in driving data collection and research

Thank you