

What will haemophilia treatment look like in 2027

A patient's perspective?

Radek Kaczmarek

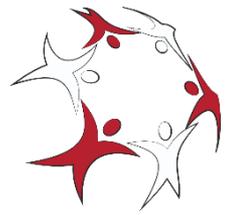
EHC Steering Committee

EHC Round Table of Stakeholders

Brussels

28 November 2017

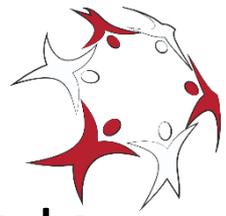
Predicting the future is a risky business...



1946: "Television won't be able to hold on to any market it captures after the first six months. People will soon get tired of staring at a plywood box every night." — Darryl Zanuck, 20th Century Fox.

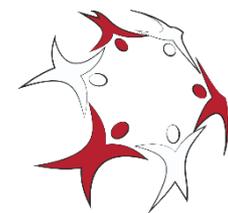
1955: "Nuclear powered vacuum cleaners will probably be a reality within 10 years." — Alex Lewyt, President of the Lewyt Vacuum Cleaner Company.

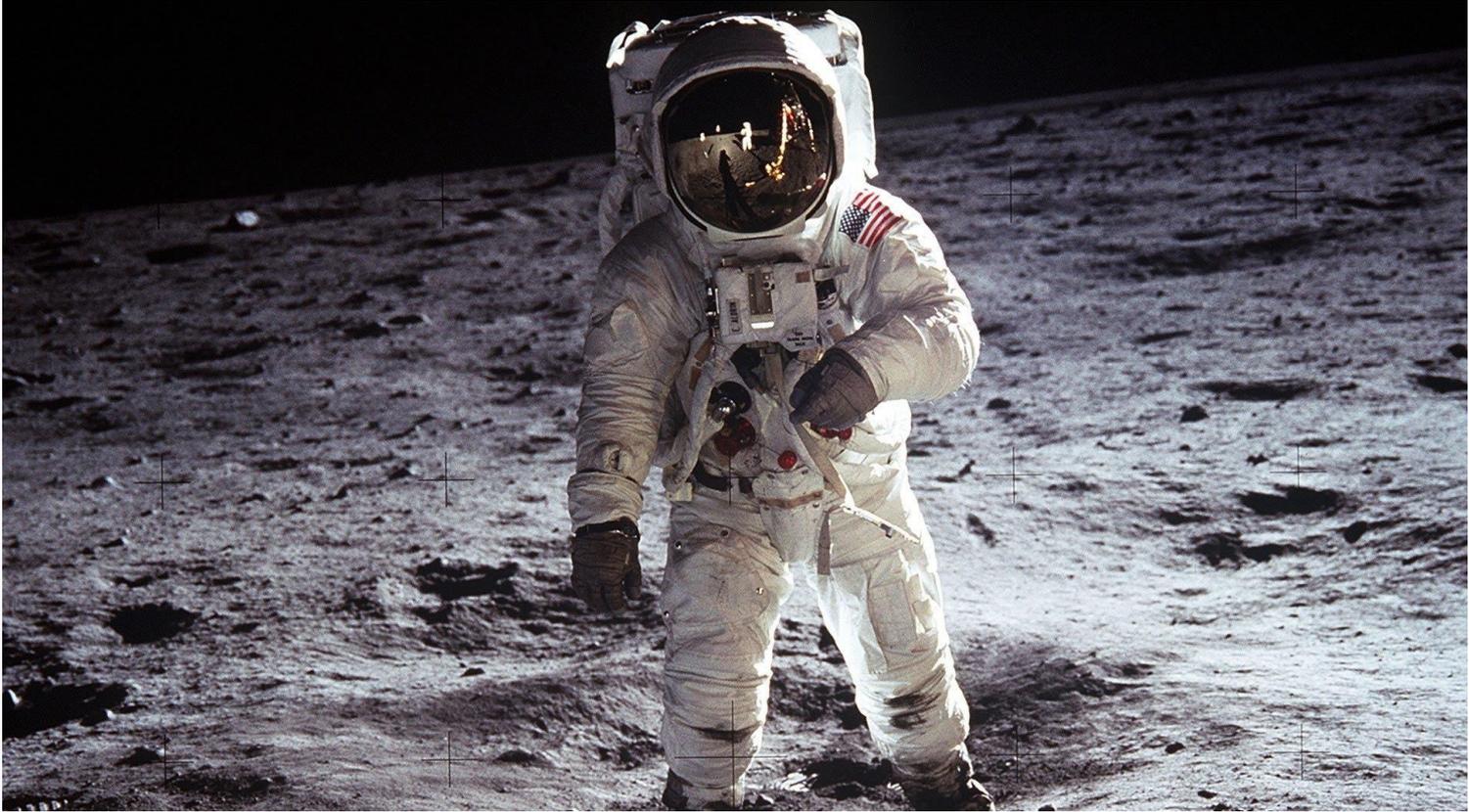
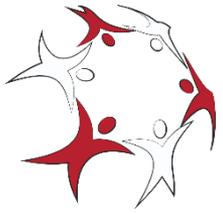
2007: "There's no chance that the iPhone is going to get any significant market share." — Steve Ballmer, Microsoft CEO.



Chris Bombardier, the first person with haemophilia to reach the summit of Mount Everest (on a longer-acting CFC).

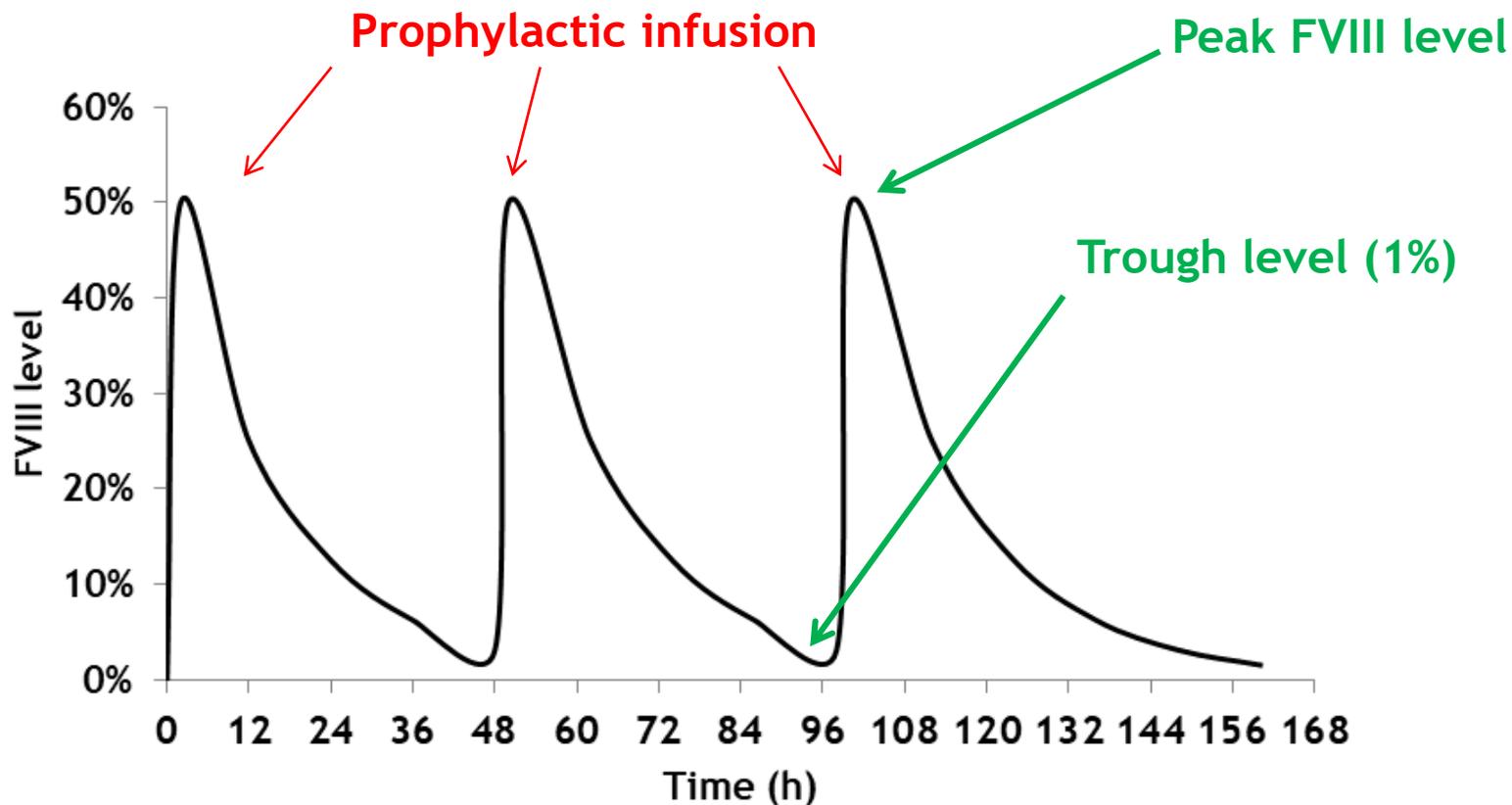






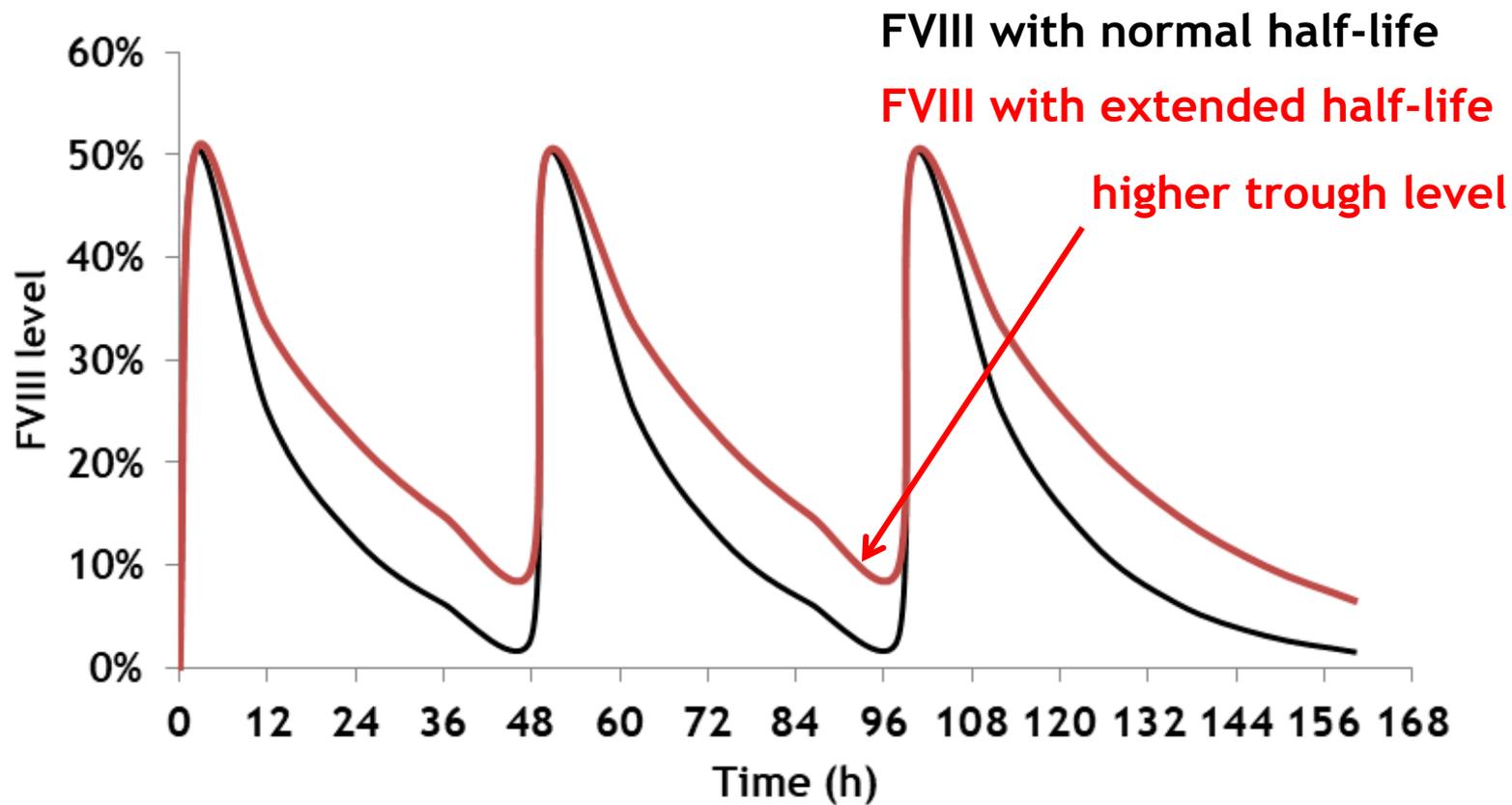


Mark Skinner in Paris, 2012 (anticipating longer-acting CFCs): what trough can we now go for?
3, 5, 15%?

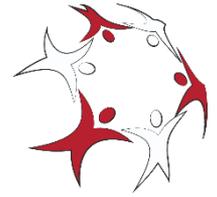




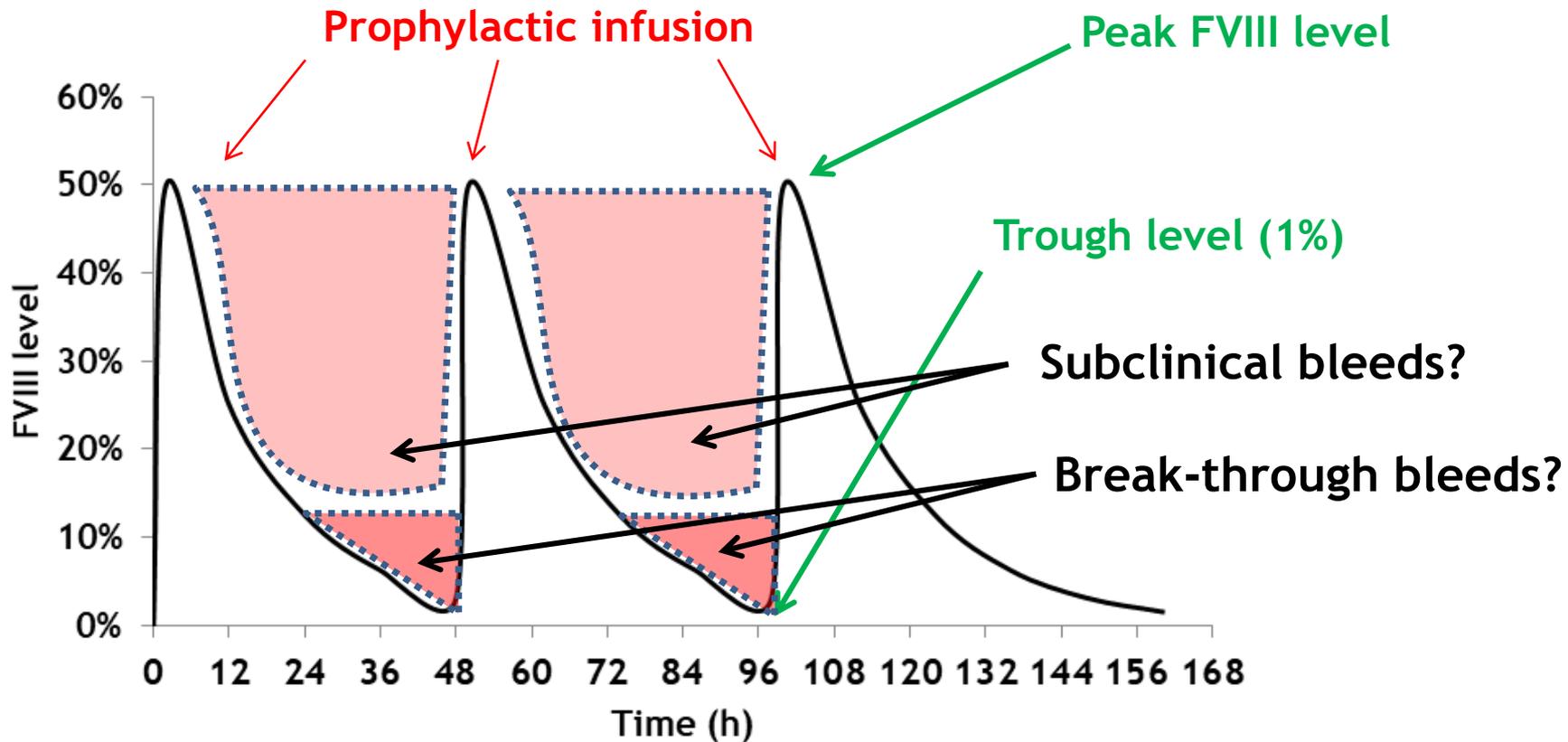
What trough is high enough?



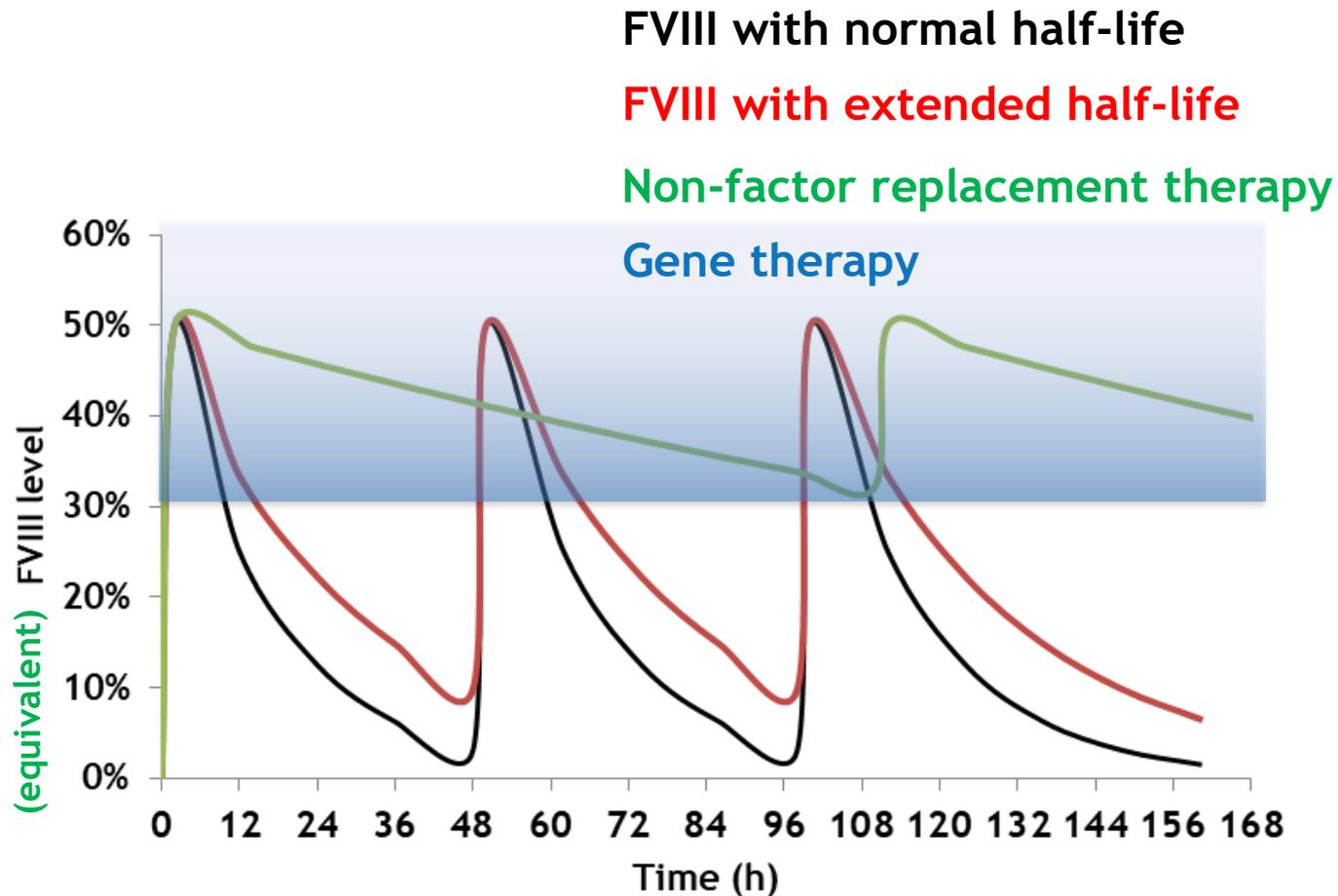
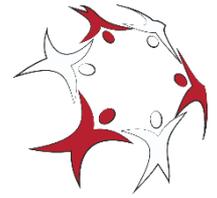
Some patients develop joint damage despite prophylaxis.



Personalisation is part of the solution, but the need for a consensus intervention giving universal protection from damage remains.



Smoothing the sawtooth:
perhaps any level lower than normal
is not good enough?



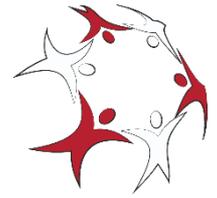
What might be the treatment landscape in 2027?



- Standard- and longer-acting CFCs will still be used routinely. Much wider use of longer-acting CFCs. Troughs unchained.



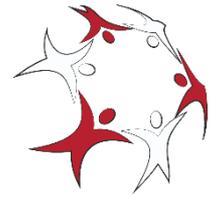
What might be the treatment landscape in 2027? (2)



- More than one non-substitutional therapy will be widely used, including drugs targeting physiological anticoagulants.



What might be the treatment landscape in 2027? (3)



- Gene therapies licensed and available to some. A few dozen people „cured”.



New questions



New challenges may emerge in the long-term management of adults with stably normalised clotting.



What Does A Healthy Aging Cat Look Like?



Conclusions



In 10 years, the haemophilia care will have been transformed in an unprecedented way.

Meanwhile, many questions will have to be answered and we have to advocate the pursuit of these answers.

As a community, we have to get comfortable with the new language of novel therapies and prepare to take firm positions.