

My experience as a hemophilia patient with gene therapy

Daan Breederveld

Hemophilia B patient

Occupational health physician

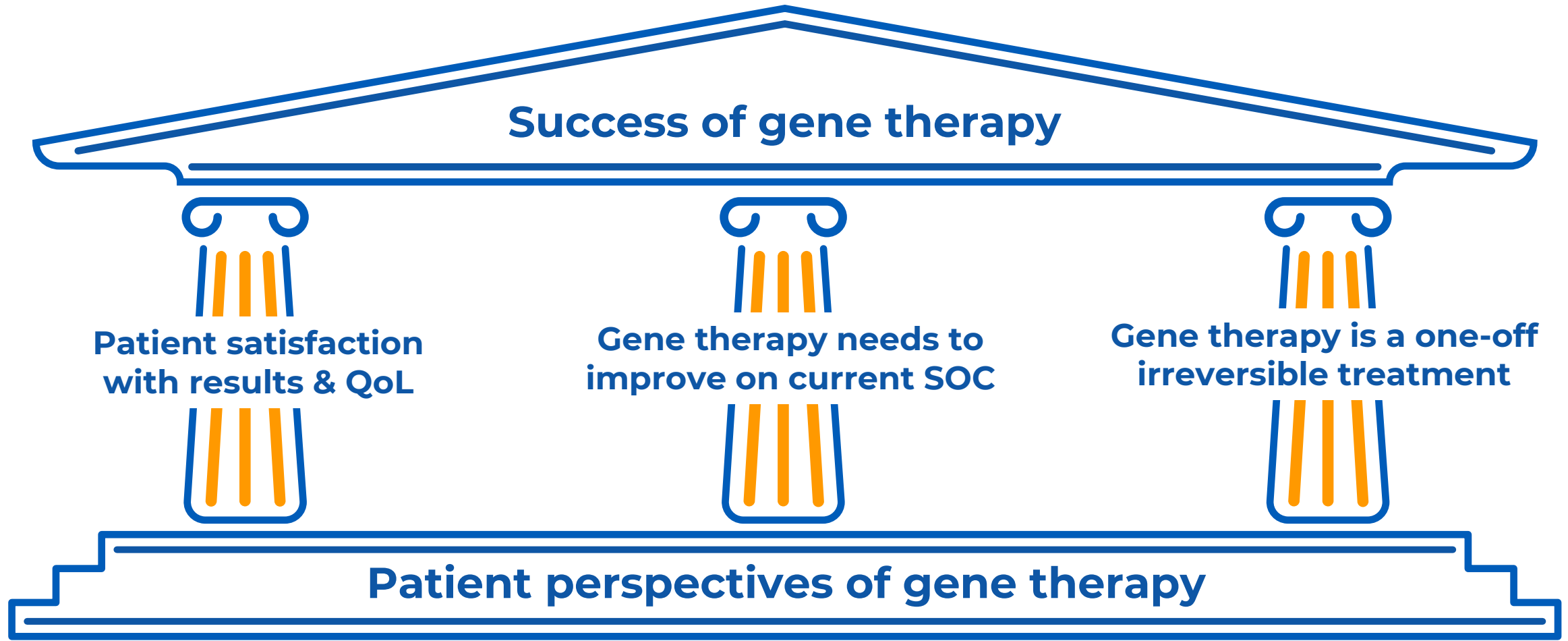


Disclosure

- Occupational Health physician, owner - OHS company in Amsterdam
- Consultancy fees CSL Behring



Why is the patient perspective on gene therapy important?



My life living with haemophilia

Daan Breederveld

- Born in Amsterdam in the 1970's
- Only child
- Mother 28 year old psychology student
- Father 29 year old medical student

- First signs: skin haematoma after venepuncture for diagnosis (right temple)



Family history

- Mother known HB carrier; FIX 60%
- Grandmother known HB carrier
- Family history of infant mortality
- Uncle died in 1947 at 10 years of age
- Uncle born with haemophilia in early 1950's



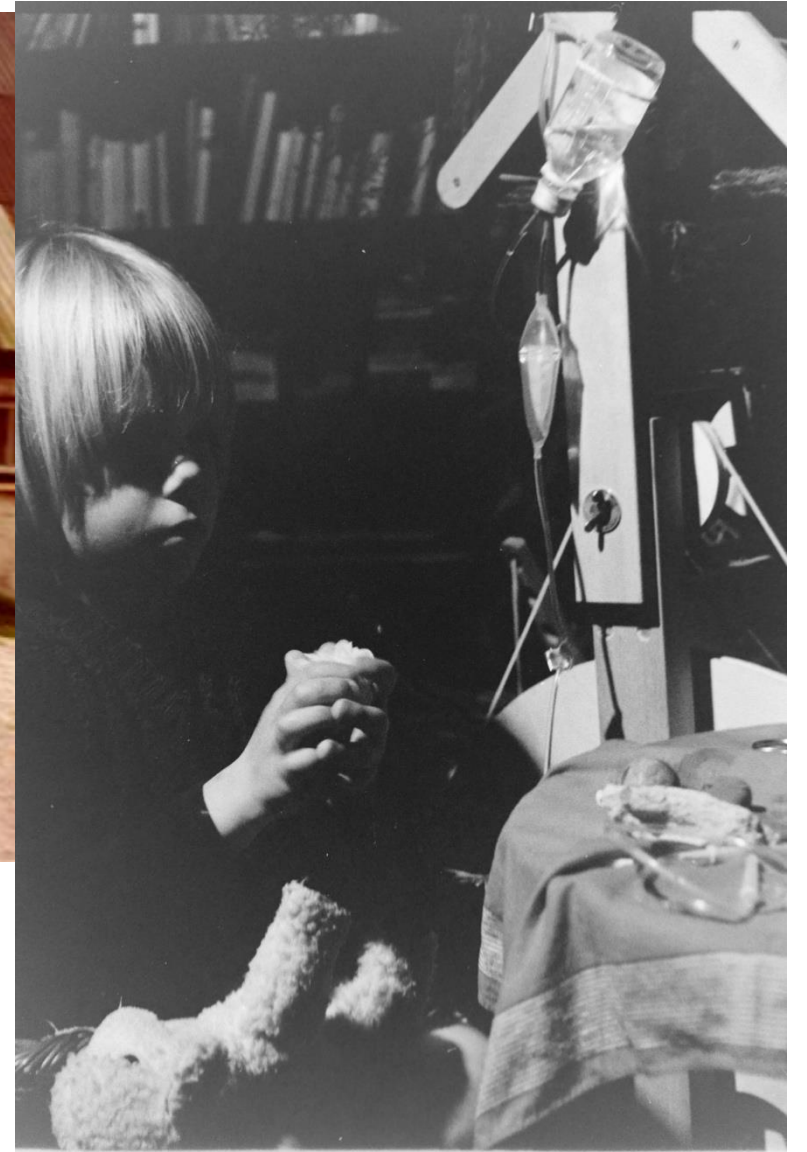
Childhood precautions



'Normal' development!



Childhood fun, direction



So why gene therapy? My personal perspective



Dreaming of a life without haemophilia dominating thoughts and influencing decisions

- Less worries about side effects, availability of product
- No more medicine logistics, hospital consultations
- Helping haemophilia research and treatment for others
- Cost of treatment, less impact on solidarity



- Relative low disease burden (or is this the disability paradox?)
- Father of three (young) children
- Negative side effects, short and/or long term (prednisone during first months/unknown)



Disease burden



Gene therapy efficacy



Gene therapy safety



Psychology

Patient perspectives of gene therapy in haemophilia

Hermans *Orphanet Journal of Rare Diseases* (2022) 17:154
<https://doi.org/10.1186/s13023-022-02313-w> Orphanet Journal of Rare Diseases

EDITORIAL **Open Access**

Haemophilia gene therapy: experiences and lessons from treated patients

Cedric Hermans*

Check for updates

Parental perspectives on gene therapy for children with haemophilia: The Exigency study

Article in *Haemophilia* · November 2020
DOI: 10.1111/hae.14188

Patient Preference and Adherence

Dovepress

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CASE SERIES

The Patient Experience of Gene Therapy for Hemophilia: Qualitative Interviews with Trial Patients

Received: 2 April 2020 | Revised: 19 October 2020 | Accepted: 20 October 2020
DOI: 10.1111/hae.14190

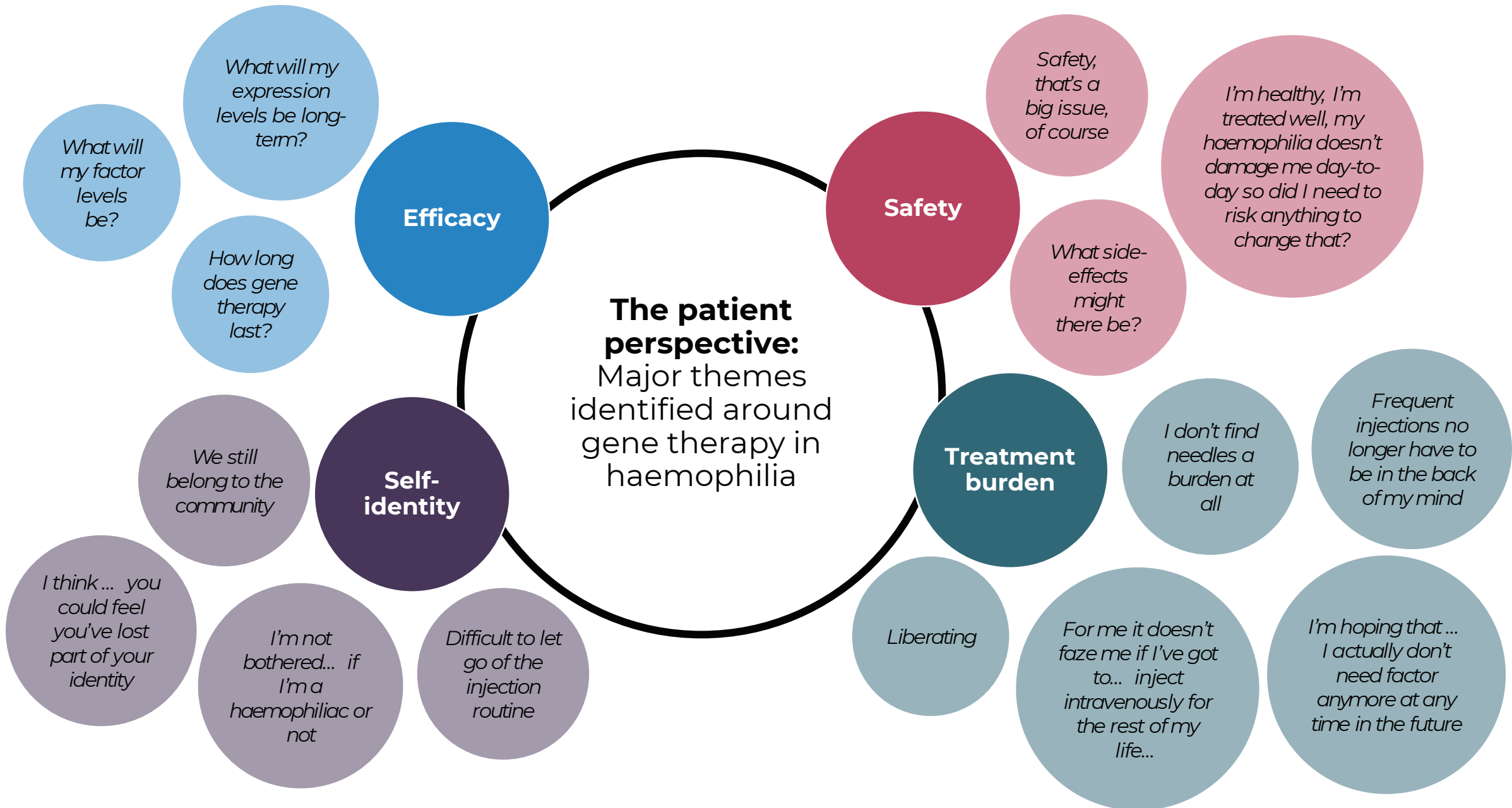
ORIGINAL ARTICLE
Clinical haemophilia

Haemophilia  WILEY

Patient perspectives regarding gene therapy in haemophilia: Interviews from the PAVING study

See discussions, stats, and author profiles for this publication at: <https://www.researchgate.net/publication/353276716>

An exploration of why men with severe haemophilia might not want gene therapy: The exigency study



How and when gene therapy entered my life

Late '90's

- Gene therapy for mice and dogs, with midterm sustainable responses

~2014

- First discussion brought up by haemophilia treatment center/ treating physician (Phase 1 study)

Gene therapy in 2019

- Dialogue with my treating physician started:
 - 1 year before therapy
 - 6 months before enrollment

Decision making process

- Analysis of the data through research of available studies
- Sharing with my family, parents and friends
 - **Family:**
 - Exploring their position, possible fears and presumptions
 - **Parents:**
 - Seeking support; mothers intuition, also valuing my father's medical knowledge and intuition
 - **Friends:**
 - Especially friends in internal medicine and research
 - Others, in using their 'laymans' approach and critical questions, prevented me from only seeing myself through doctors' eyes only

Peri-administration period

- **Excitement:**
 - In my case 'healthy tension'; but also reckon with an anxious patient and be prepared to handle this
- **No way back:**
 - As soon as I entered the room, I realised that I was going to have the therapy. Leaving before or during administration would have (partially unknown) consequences
- **Patien(t)ce:**
 - Monitoring side effects, staying in the room for 1 hour after administration; staying in the hospital during at least 4 hours.



Follow up: My first year and now

First days –
weeks

Strange feeling of 'change' within

After 22 days

Slipped on concrete floor, landed left hip first and **no signs of haematoma**
- Could not believe this to be true, but several other smaller incidents provided proof of concept

Focus on percentages

March 2020

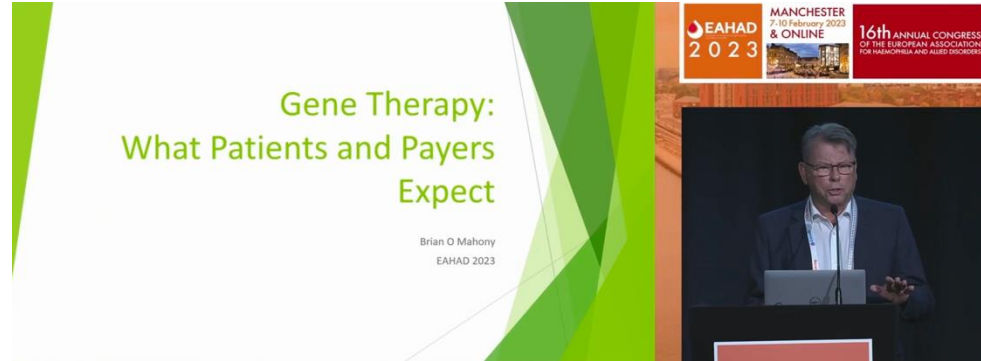
Have not been using any FIX product since gene therapy; I realise that it might actually be happening in my life

COVID-19.....

Today

My daughter, 9 yo., carrier and haemophilia B patient showed disappointment since my FIX levels now topped hers...

Other perspectives



What Patients Expect From Clinicians and Centres

- ▶ Key is setting and managing expectations
- ▶ **Realistic conversations with the relevant clinicians over a period of time.**
- ▶ Their experience of living with haemophilia, joint status, ABR, quality of life, activities and goals
- ▶ Clear understanding of the knowns and unknowns
- ▶ Understanding of monitoring and follow up requirements and long term duration of monitoring
- ▶ Understanding of lifestyle implications- alcohol, exercise
- ▶ Understanding of what Gene addition Therapy is and what it is not

What Patients Expect From Clinicians and Centres

- ▶ Objective view
- ▶ Setting realistic expectations- what their ideal outcome would be and clear understanding of range of outcomes possible and lack of predictability for each outcome
- ▶ What factor expression would they like
- ▶ Possibility of achieving no significant factor expression
- ▶ What duration of expression would they like
- ▶ Willingness to take steroids- for how long
- ▶ Theoretical risk of cancer

- ▶ **Solid arrangements to manage the journey**
- ▶ Clear co-ordination between their Hub and Spoke centres- who does what and when
- ▶ Arrangements for regular phlebotomy – at hub, spoke, local hospital, at home
- ▶ Logistical arrangements to ensure PWH can comply with all monitoring requirements- dovetail with work, college - timing

Life after being part of a gene therapy trial

