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Challenges and Benefits of New Treatments

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Haemophilia Treatments - Past

Cryoprecipitate

Plasma Derived Factor – AHF

Treatment for bleeds only

Haemophilia Treatment- Present

- Recombinant Factors - Standard Half Life
- Introduction of prophylaxis & home therapy

Benefits:

- Home administration & less frequent admissions to hospital
- Able to tailor the administration around high risk activities
- Treat bleeds quickly

Challenges:

- Learning skills for IV access or implanted central venous access device (CVAD – "port")
- Risk of infection with CVADs
- Risk of inhibitor development
- Frequent infusions – causing damage and scarring to veins leading to poor compliance
- Impact on family life and daily routines
- Financial burden
- Didn't address the long term consequences of joint bleeds and damage already existing.

Haemophilia Treatments – Present

Extended Half life products

Pharmacokinetic (PK) studies to assess half life became accessible with reduced samples required

Applications for families in the home to be able to monitor predicted levels

Benefits:

- Frequency in administration reduced
    - Particularly haemophilia B
- PK studies allowed confidence in tailoring frequency of dosing
- Access to applications i.e. MYWAPPs allowed reassurance and planning of activities for parents

Challenges:

- Still relies on IV administration and Implanted Venous access devices

Haemophilia Treatment – Present

- Recombinant humanised bi-specific antibody
  - Emicizumab (Hemlibra)
  - Mimics the action of FVIII

Benefits:

- Long half life – less frequent administration – weekly, fortnightly or monthly
- Provides stable rather than fluctuating levels with factor replacement – removes the need for planning ahead with daily activities
- Severe and moderate haemophilia A with or without inhibitors
- Subcutaneous administration

Challenges:

- Does not produce the high factor levels required for high risk activities, contact sports and trauma injuries
- Limited data on surgical management and break through bleeding
- Factor replacement is still required
- Learn new technique of administration
- Fear of the unknown
- Will it work – How will I know it is working?
- Not available for Mild Haemophilia A or other bleeding disorders

Haemophilia Treatment-Future

- Gene therapy
- Treatments that Correct the coagulation imbalance – prevents antithrombin production and enhances thrombin generation
- Treatments that Target tissue factor pathway inhibitor leading to stabilisation of clots.
- How do you choose?
  - Shared decision making

Our Aim

- To support children and families to have a quality of life that they desire, maintain pristine joint health whilst advances in technology and scientists develop new improved treatment, cures are developed and made accessible by governments.

- If we don't, accessing a cure in gene therapy will not improve the quality of life as it will not undo the damage to joints and consequential pain and limitations in function will be lifelong.

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