

John Dempster  
Lead Clinical Nurse Specialist (CNS) Immunology  
Bart's Health NHS trust  
London, UK

# HAE – patients experience in clinical trials



# Experience with Lanadelumab as an IMP

- 4 patients on open-label Dyax trial at our centre
- Patient response and experience - a big thumbs up!
- Let me share their experience with you

# Patient 1

- Reduces the stress of **unpredictability**
- Regular, severe abdominal attacks
- Access only to Icatibant before Dyax
- Things change HAE patient day
- Dyax trial and then open label
- 2-3 attacks weekly (severe) Vs every 6 weeks(mild)

- Life changing – now able to function at almost 100%
- Now confident to accept leadership role in retreat centre
- Injection:
  - Would like pre-filled pen, challenging for some
  - Stings a bit, no more than Icatibant
  - One training session for treatment experienced patients?

# Patient 2

- Most severe patient
- Started injecting C1 33 years ago
- Dyax open label – 1 attack in 12 months!
- Almost forgets she has HAE
- Can commit to things in future, holidays etc
- Can play with grandchildren

- Wish list for licensed product:
  - Pre-filled syringe
  - No fridge
  - App on phone to record and bleep
  - SMS reminder via homecare
  - Only 1 training session for tx experienced, more if required

# Patient 3

- Previously on Oxanrolone, on-demand Icatibant and Ruconest
- “Superb”.Attack free for 12 months
- Less painful than Icatibant
- 1 attack –treated with Ruconest, hasn’t lost IV skill
- Hasn’t got to worry about attacks – freedom!

- Licensed product:
  - 1 vial to draw up or pre-filled injection
  - Easier transfer device
  - Reminder on fridge
  - Document as soon as given



# Patient 4

- “Miracle drug”
- Relief not having to inject Cinryze twice a week due to other co-morbidities
- Breakthrough attacks much less and milder
- A phone/text reminder good
- Licensed treatment by HCP

# Role for lanadelumab in future

- Hopefully a major part in LTP management - Lana Vs C1inh Vs oral tx
- Who gets it?
- Economic model/dosing regimen/schedule
- UK commissioning policy on HAE

# BCX7353-204

- 2 patients – open label
- Now one tablet a day (with food)
- Patient 1 – started May 2018. Had 1-2 attacks per week, now approx 1 per month. No side effects
- Patient 2 – Started April 2018. Had 2-3 attacks per week, now approx 1 attack every 2 weeks. No side effects.

Thank you