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 Cinrzye 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