

# Update on access to Omaveloxolone for adults in the UK with FA

23<sup>rd</sup> May 2025

Webinar with  realise advocacy

# ATAXIA

# Welcome

Sue Millman, CEO Ataxia UK

## Agenda

**12:00** Welcome & Housekeeping

**12:05** Presentation from Realise Advocacy

**12:10** Presentation from Ataxia UK

**12:20** Questions and Answers to Panel

**12:55** Closing remarks from Chair

**13:00** Close

## Housekeeping

- *Please stay on mute unless asked to speak*
- *Please post questions using Q&A feature*
- *Recording the meeting – please turn off your camera if you don't wish to be seen*

## Goals

- Update FA community - access of Omaveloxolone on the NHS in UK
- Inform actions Ataxia UK are taking and how community can help
- Opportunity to ask questions and share thoughts

# Omaaveloxolone overview

- ❑ Omaaveloxolone (Skyclarys) is a new treatment that was approved in the US (2023) and Europe (2024) for treating Friedreich's ataxia in people 16 years and over
- ❑ On 23rd April 2025 it was approved also by the UK regulator MHRA for treating Friedreich's ataxia in people 16 years and over
- ❑ The manufacturer is Biogen. They acquired from Reata pharmaceuticals in Sep 2023
- ❑ Biogen announced plans for a trial in children with FA with three UK sites

# Health Technology Assessment in the UK

Lindsay Weaver – Realise Advocacy



# The NICE process

## Medicines – Highly Specialised Technologies and Technology Appraisals

Week 49  
Guidance  
Published  
Or Week 40

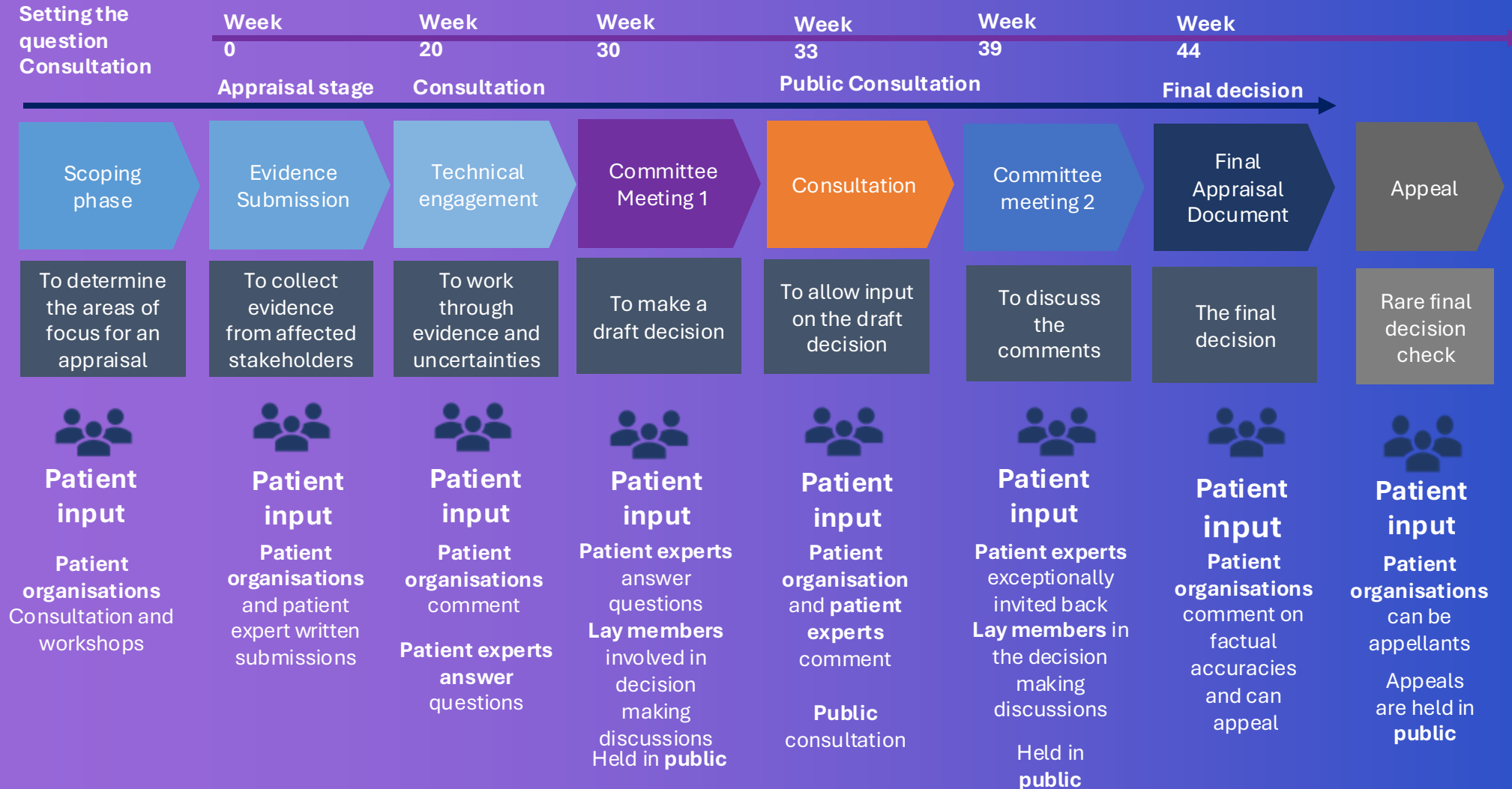
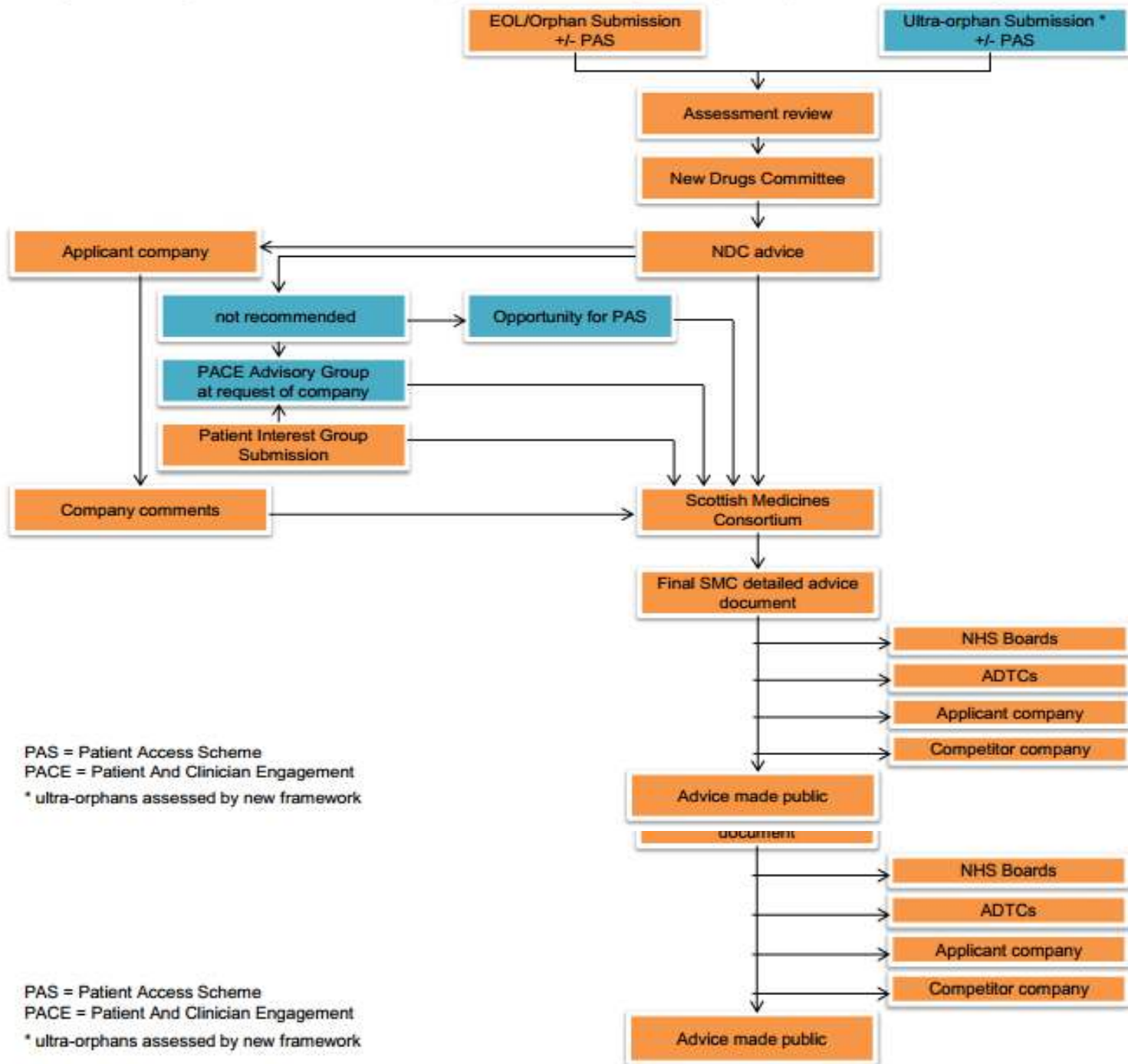


Diagram 1 – Integration of PACE into SMC process for EoL, orphan/orphan-equivalent and ultra-orphan medicines



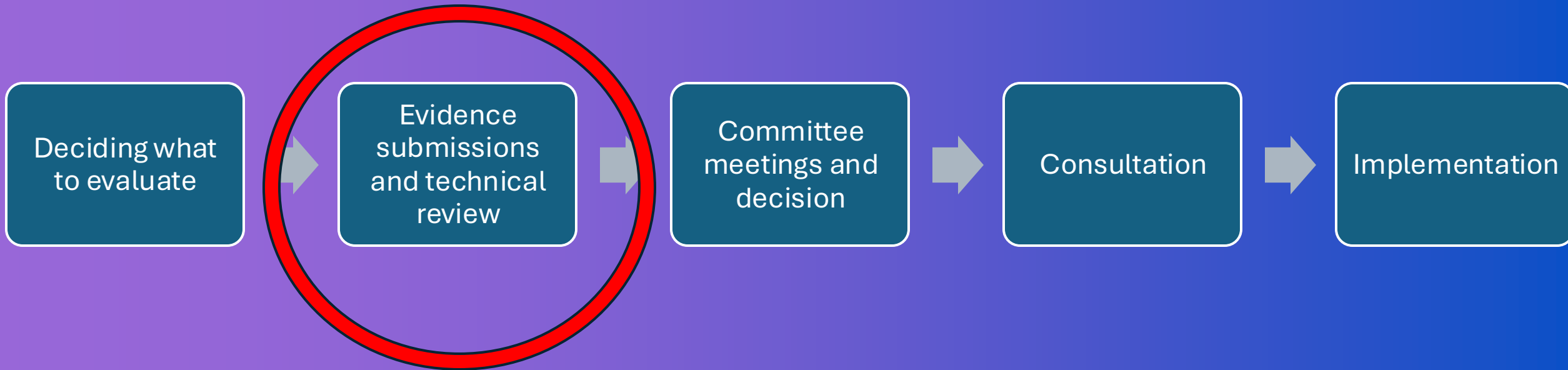
## The SMC process - Scotland

Assessment timeline usually 18 weeks.

PACE meeting can add 1-3 months to timeline.

<https://scottishmedicines.org.uk/how-we-decide/interim-acceptance-decision-option/>

# Omaceloxolone update



- Biogen have withdrawn from the NICE process as NICE has declined to hold a committee meeting. This can happen:
  - If the manufacturer does not submit evidence or withdraws their submission
  - If NICE is not satisfied that the evidence submission is adequate for the committee to make a decision
  - When there is no plausible potential for the treatment to be cost-effective and the manufacturer is unwilling to offer a patient access scheme or if commercial discussions with NHS England are not possible.

# What has Ataxia UK done in NICE process

Dr Julie Greenfield, Director of Research

## □ NICE process

- Ensured patient involvement in process
- Submitted evidence for scoping process
- Attended NICE scoping meeting (Aug 2024)
- Gathered evidence for NICE submission:
  - Case studies from people affected by FA
  - Real world evidence of use of Omav
- Submitted evidence to NICE (early Jan 2025 deadline)
- Dialogue with NICE post withdrawal of Biogen from evaluation

# Ataxia UK next actions: advocacy strategy communication and collaboration

- Speaking with relevant stakeholders:
  - FA community
  - Dialogue with NICE - what is needed to resume NICE process and have a chance to discuss submission in Evaluation Committee meeting
  - Ataxia Centre clinicians and other neurologists
  - Genetic Alliance UK, other rare disease charities with experience of NICE
  - Biogen

# Ataxia UK next actions: advocacy strategy

## Biogen

- Continue dialogue with Biogen
  - Keep them informed of our plans
  - Encourage Biogen to act quickly on their confirmed intention to resubmit by investigating potential for negotiation with NICE, and support this effort
  - Explore all potential routes for early access

# Ataxia UK next actions: advocacy strategy campaigning

- Writing to Secretary of State for Health & Social Care asking for a meeting
  - Highlight urgent need and unfairness
  - Early access to Omav
  - Changes in evaluation system for rare conditions
- Ask ataxia community to sign letter to Minister to add weight and forward to MPs

# Ataxia UK next actions: advocacy campaign

## Awareness

- Raise our issue with MPs by attending All Party Parliamentary Group for rare diseases meeting - June/July

Subject of meeting:

- Report from LifeArc/Genetic Alliance on difficulties with access to medicines in rare diseases
- Ataxia UK will present Omaveloxolone as a case study
- Attend launch event for report from LifeArc/Genetic Alliance on difficulties with access to medicines
- Explore opportunities for awareness in the media

# Ataxia UK next actions: Scotland

- ❑ Work on access for people with FA in Scotland
  - ❑ Biogen plan to submit to SMC in June
  - ❑ Ataxia UK is a registered stakeholder with SMC
  - ❑ Contribute to evaluation of Omav in Scotland by SMC
  - ❑ Support and advocate for patients to get early access via the established scheme in Scotland

**Our panel will now take questions from audience members. For your comfort, please be reminded of the following housekeeping guidelines:**

- Please post questions in the chat
- Please stay on mute unless asked to speak
- Questions not answered at the meeting will be responded to by email.

# Q&A

# ATAXIA