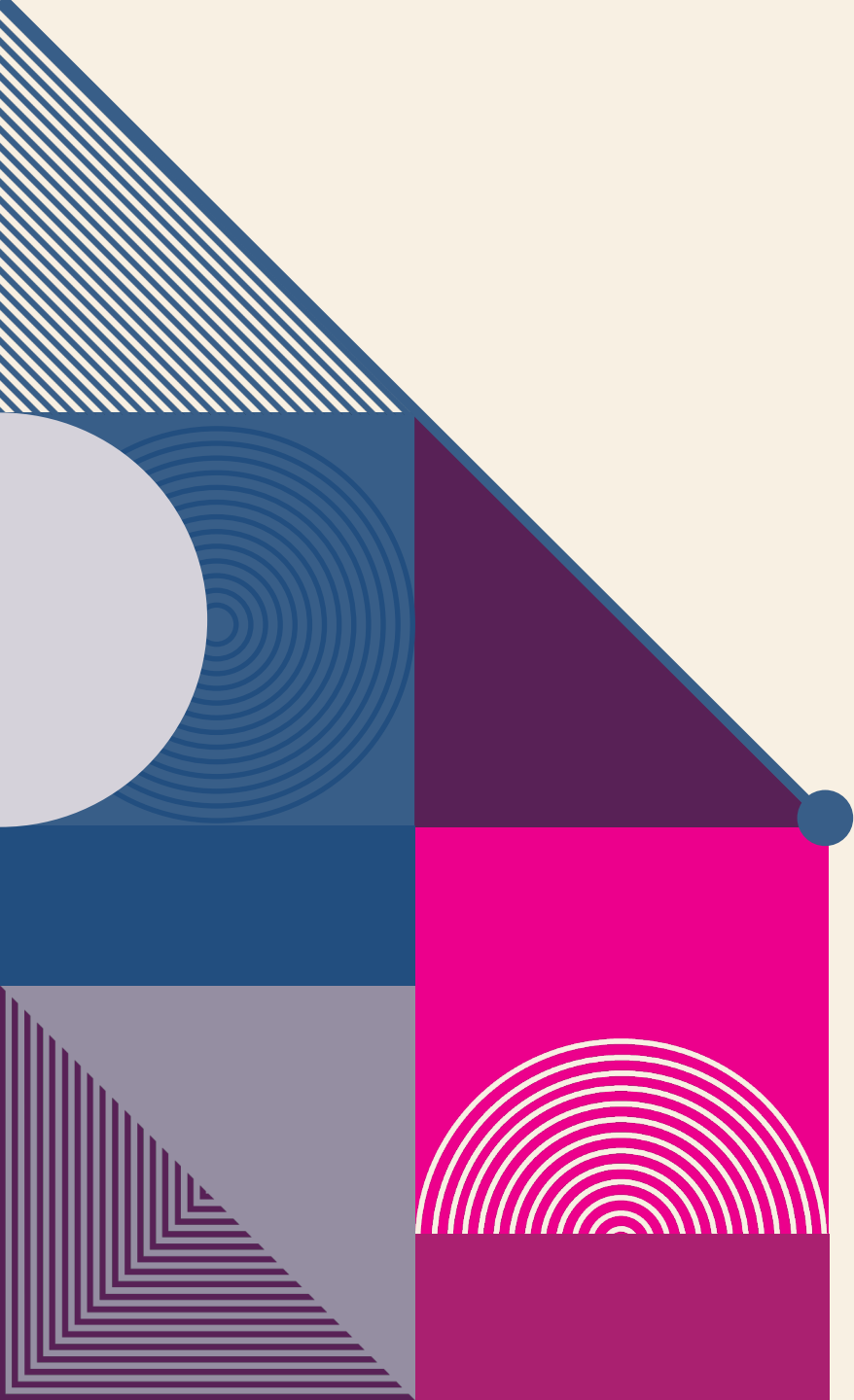




# ALS & CLINICAL STUDY - A PATIENT'S PERSPECTIVE



# AGENDA

Short Bio of the Speaker

Diagnostical Delay

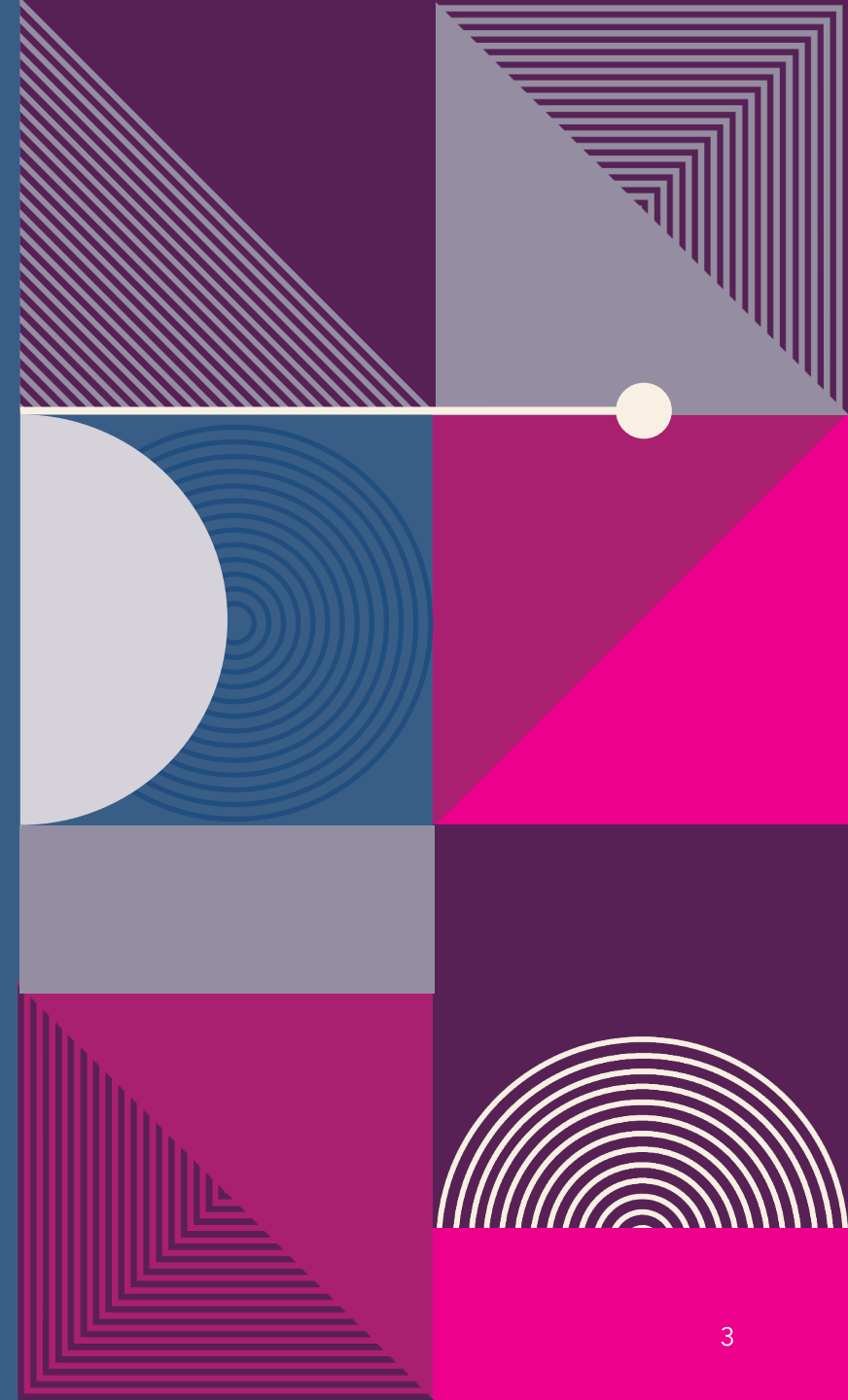
Clinical Study - Phase I - Experiences

Conclusion

Expression of Gratitude and Hope

# SHORT BIO OF THE SPEAKER - PATIENT

- Tony Groessens
- ° March 26<sup>th</sup>, 1966 – 59 years old
- Hereditary form of ALS – FUS mutation
- Family : Wife – 2 Children
- Still active professionally – CPA





# DIAGNOSTICAL DELAY

- First, non-identified symptoms : 10-11/2023
- From 12/2023 - 06/2024 :
  - Multiple CT and MRI scans
  - Medical visits and assessments
- Mid 07/2024
  - Functional Measurements at UZ Leuven
  - Appointment with Prof. Van Damme
  - Same day : provisional assessment
- End of 08/2024 - final diagnosis after DNA analysis

**=> Diagnosis takes too long**

# CLINICAL STUDY PROPOSAL

- Possibility and basic information during 07-08/2024
- Motivation :
  1. Future of my children given the ALS type
  2. Advancement of science is crucial
  3. Placebo should not be a deterrent but remains conflicting from a patient's perspective
    - Scientific good practice
    - In case of physical decline => after 12 months intervention in case of placebo
  4. What is the alternative ?

# CLINICAL STUDY – PHASE I – EXPERIENCES (1)

## Timeline

- Information to patient-candidate relative to the study (09-11/2024)
- Screening day (14/11/2024)
- First treatment – Extensive monitoring (9-10/12/2024)
- Second and Third treatment (9/01/2025 – 6/03/2025)
- Next/Fourth treatment (26/05/2025)

# CLINICAL STUDY – PHASE I – EXPERIENCES (2)

## Medical treatment and support

### 1. Examination

- Professional approach
- Structured and recurring query lists, observations (breathing, limb strength, body functions,...) and blood test
- Adequate information on the examination topics

### 2. Lumbar Puncture

- Performed with due professionalism and care
- Adequate oral information on the actions performed during the procedure

# CLINICAL STUDY – PHASE I – EXPERIENCES (3)

## Medical treatment and support

### *3. Adequate post-treatment follow-up*

- Post-treatment information gathering on patient's condition by the medical staff
- Assistance with home assessment

### *4. Professional and caring/helpful medical staff*

### *5. Appropriate logistical setting – clinical facilities*



# CONCLUSION

- Diagnostical delay should be limited as much as possible. Timely referral to the adequate medical professional is needed.
- The IONIS study 363 - Phase I is well managed and structured. The medical accompaniment and support are more than satisfying. Excellent work.

# GRATITUDE & HOPE

Thanks to :

1. Prof. Van Damme and his medical staff for the care and the follow-up
2. IONIS for their research and the possibility to participate in the study

Hope :

- Continuous R&D and investments from pharmaceutical companies and
- Funding of research by the public authorities in order to irradiate devastating diseases



***“OPTIMISM IS A MORAL  
DUTY”***



**THANK YOU**

Tony Groessens

**Q & A**