In collaboration with its Scientific Board, and in partnership with DéfiScience (the National Network for Intellectual Disabilities), the Fragile X France association (www.xfra.org) is proud to announce its next international workshop dedicated to the Fragile X Syndrome and premutation associated conditions.

This collaborative working meeting aims to connect researchers & clinicians working on Fragile X syndrome in order to consolidate or bring out new therapeutic leads.

Given the still uncertain health context, the Workshop on Fragile X is organized online.

**PROGRAM**

**THURSDAY MARCH, 18TH**

**10 a.m. to 1 p.m. : Clinical update** (Chairmen : Dr Charles - Pr Lesca - Pr Anheim)
Clinical genetics in 2020 *(Dr Delphine Héron)*
Clinical follow up for Fragile X *(Pr Vincent des Portes)*
Impact on daily life *(Christine Kieffer - Fragile X France)*
Impact on daily life : DIXIT study *(Dr Aurore Curie & Dr Clémentine Fort)*
FXTAS *(Dr Perrine Charles)*
FXPO *(Pr Anne Bachelot)*

**2 p.m. to 5 p.m. : Physiopathology of Fragile X Syndrome** (Chairmen : Dr Curie - Dr Moine)
FMRP : function, molecular targets, mouse models *(Dr Hervé Moine)*
Neurobiology of FXTAS and therapeutic approaches *(Dr Nicolas Charlet Berguerand)*
Neurobiology of Fragile X : revolution of iPS and organoides *(Dr Peng Jin)*
Modeling Fragile X Syndrome with iPSCs *(Dr Cecilia Laterza)*

**FRIDAY MARCH, 19TH**

**10 a.m. to 1 p.m. : Therapeutics of Fragile X Syndrome** (Chairmen : Dr Héron - Pr des Portes)
What therapeutic trial plans ? *(Dr Aurore Curie)*
Efficacy judgment criteria in Fragile X *(Dr Aurore Curie & Dr Perrine Charles)*
Identification of non-invasive clinical markers of FXS *(Dr Sylvain Briault)*
Fragile X : 30 years of research *(Pr Jean-Louis Mandel)*
Fragile X : 30 years later : satisfactions, worries, expectations *(Christine Kieffer - Fragile X France)*

**2 p.m. to 5 p.m. : Innovative therapies, drug candidates** (Chairmen : Dr Jacquemont - Pr Mandel)
Therapeutic trials stopped & in progress, what lessons ? *(Dr Sébastien Jacquemont)*
DGKK gene therapy for Fragile X treatment : the GETEX project *(Dr Hervé Moine)*
Therapeutic Potential of CRISPR/Cas9 Mediated Deletion of CGG repeats for FMR1 Gene Reactivation in Fragile X Syndrome *(Dr Daman Kumari)*
Involvement of Phosphodiesterase 2A Activity : a new treatment for Fragile X Syndrome and other forms of neuronal development disease *(Dr Barbara Bardoni)*

**5 p.m. to 5:15 p.m. : Conclusion**

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**INFORMATIONS**

**PUBLIC**
Clinicians & Researchers
All discussions in English

**DATES**
March 18th & 19th, 2021
From 10 a.m. to 1 p.m.
And from 2 p.m. to 5 p.m.

**INSCRIPTIONS**
Registration required
Possible until march 17th, 2021
www.xfra.org
www.defiscience.fr

**PRICES**
Free registration

**CONTACT**
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