



INTERNATIONAL WORKSHOP FRAGILE X & ASSOCIATED CONDITIONS MARCH 18TH & 19TH 2021 - PARIS, FRANCE



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 will aim to connect **researchers & clinicians** working on Fragile X syndrome in order to **consolidate or bring out new therapeutic leads**.

PROGRAM

THURSDAY MARCH 18TH, 2021

4 p.m. - 5 p.m. : Opening

Fragile X : 30 years later : satisfactions, worries, expectations (*Fragile X France*)

X fragile : 30 years of research (*Jean-Louis Mandel*)

5 p.m. - 8 p.m. : Clinical update

Clinical genetics in 2020 (*Delphine Héron*)

Clinical follow up for Fragile X (*Vincent des Portes*)

Impact on daily life (*Fragile X France*)

Impact on daily life : DIXIT study (*Aurore Curie & Clémentine Fort*)

FXTAS (*Perrine Charles*)

FXPOI (*Anne Bachelot*)

8 p.m. - 10 p.m. : Informal dinner cocktail

FRIDAY MARCH 19TH, 2021

8:30 a.m. - 12:30 p.m. : Physiopathology of fragile X syndrome

FMRP : function, molecular targets, mouse models (*Hervé Moine*)

Neurobiology of fragile X : revolution of iPSC and organoids (*Peng Jin*)

Modeling Fragile X Syndrome with iPSCs (*Cecilia Laterza*)

Neurobiology of FXTAS and therapeutic approaches (*Nicolas Charlet Berguerand*)

Round table

12:30 p.m. - 1:30 p.m. : Lunch

1:30 p.m. - 3:30 p.m. : Therapeutics of fragile X syndrome

DGKK gene therapy for Fragile X treatment : the GETEX project (*Hervé Moine*)

Randomized trials vs placebo : interest and limits (*Aurore Curie*)

Efficacy judgment criteria in fragile X (*Aurore Curie & Perrine Charles*)

Why and how to participate in a therapeutic trial ? (*Ségolène Gaillard - Kids France*)

4 p.m. - 6:30 p.m. : Innovative therapies, drug candidates

Therapeutic trials stopped and in progress : what lessons ? (*Sébastien Jacquemont*)

Therapeutic Potential of CRISPR/Cas9 Mediated Deletion of CGG repeats for FMR1 Gene Reactivation in Fragile X Syndrome (*Daman Kumari*)

Identification of non-invasive clinical markers of FXS (*Sylvain Briault*)

Involvement of Phosphodiesterase 2A Activity : a new treatment for Fragile X syndrome and other forms of neuronal development disease (*Barbara Bardonni*)

INFORMATIONS

PUBLIC

Clinicians & Researchers

All discussions in English

DATES

March 18th, 2021,
from 4 p.m. to 10 p.m.

March 19th, 2021,
from 8:30 a.m. to 6:30 p.m.

REGISTRATIONS

www.xfra.org

www.defiscience.fr

LOCATION

MAS

Salle Emile Lafon

10 - 18 Rue des Terres au Curé

75013 Paris

PRICES

120 euros for both days, Thursday evening's dinner and Friday's lunch included

CONTACT

For any further information or comments, please contact us at :

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