



INTERNATIONAL WORKSHOP  
FRAGILE X & ASSOCIATED CONDITIONS  
OCTOBER 8TH & 9TH 2020 - PARIS, FRANCE



In collaboration with our **Scientific Board**, and in partnership with **DéfiScience** (the National Network for Intellectual Disabilities), the **Fragile X France association** ([www.xfra.org](http://www.xfra.org)) is proud to announce our next **international workshop for researchers & clinicians** dedicated to the **Fragile X Syndrome and premutation associated conditions**.

This collaborative working meeting will aim to connect professionals working on Fragile X syndrome in order to **consolidate or bring out new therapeutic leads**.

#### PROGRAM

##### THURSDAY OCTOBER 8TH, 2020

###### 4 p.m. - 6 p.m. : Opening

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

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

###### 6 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*)

###### 8 p.m. - 9 p.m. : Informal dinner cocktail

##### FRIDAY OCTOBER 9TH, 2020

###### 9 a.m. - 1 p.m. : Physiopathology of fragile X syndrome

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

Neurobiology of fragile X : revolution of iPS and organoides (*Peng Jin*)

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

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

Round table

###### 1 p.m. - 2 p.m. : Lunch

###### 2 p.m. - 5 p.m. : Therapeutics of fragile X syndrome

Preclinical studies : how to have a critical analysis ? (*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, Fragile X France*)

###### 5 p.m. - 7 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*)

BKCa channel opener (*Sylvain Briault*)

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

#### INFORMATION

##### PUBLIC

Clinicians & Researchers

##### DATES

October 8th, 2020, from 4 p.m. to 8 p.m.

October 9th, 2020, from 9 a.m. to 7 p.m.

##### REGISTRATIONS

[www.xfra.org](http://www.xfra.org)

[www.defiscience.fr](http://www.defiscience.fr)

##### LOCATION

Institut du Cerveau et de la Moelle

Hôpital de la Pitié Salpêtrière

47 Boulevard de l'Hôpital

75013 Paris

##### PRICES

120 euros for the two days, including dinner on Thursday evening and lunch Friday

##### CONTACT

For any further information, please contact us at :

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