



INTERNATIONAL E-WORKSHOP FRAGILE X & ASSOCIATED CONDITIONS MARCH 18TH & 19TH 2021 - ONLINE



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**)

FXPOI (**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 Bardonni**)

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

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

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

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