



*brain sciences*



*Special Issue Reprint*

## **Towards Mechanism-based Treatments for Fragile X Syndrome**

[www.mdpi.com/books/reprint/1554](http://www.mdpi.com/books/reprint/1554)

Edited by  
Daman Kumari  
Inbal Gazy

ISBN 978-3-03921-505-8 (Softback)  
ISBN 978-3-03921-506-5 (PDF)



It has been more than 25 years since the identification of the FMR1 gene and the demonstration of the causative role of CGG-repeat expansion in the disease pathology of fragile X syndrome (FXS), but the underlying mechanisms involved in the expansion mutation and the resulting gene silencing still remain elusive. Our understanding of the pathways impacted by the loss of FMRP function has grown tremendously, and has opened new avenues for targeted treatments for FXS. However, the failure of recent clinical trials that were based on successful preclinical studies using the Fmr1 knockout mouse model has forced the scientific community to revisit clinical trial design and identify objective outcome measures. There has also been a renewed interest in restoring FMR1 gene expression as a possible treatment approach for FXS. This special issue of Brain Sciences highlights the progress that has been made towards understanding the disease mechanisms and how this has informed the development of treatment strategies that are being explored for FXS.



Order Your Print Copy  
You can order print copies at  
[www.mdpi.com/books/reprint/1554](http://www.mdpi.com/books/reprint/1554)

MDPI Books offers quality open access book publishing to promote the exchange of ideas and knowledge in a globalized world. MDPI Books encompasses all the benefits of open access – high availability and visibility, as well as wide and rapid dissemination. With MDPI Books, you can complement the digital version of your work with a high quality printed counterpart.



### **Open Access**

Your scholarly work is accessible worldwide without any restrictions. All authors retain the copyright for their work distributed under the terms of the Creative Commons Attribution License.



### **Author Focus**

Authors and editors profit from MDPI's over two decades of experience in open access publishing, our customized personal support throughout the entire publication process, and competitive processing charges as well as unique contributor discounts on book purchases.



### **High Quality & Rapid Publication**

MDPI ensures a thorough review for all published items and provides a fast publication procedure. State-of-the-art research and time-sensitive topics are released with a minimum amount of delay.



### **High Visibility**

Due to our global network and well-known channel partners, we ensure maximum visibility and broad dissemination. Title information of books is sent to international indexing databases and archives, such as the Directory of Open Access Books (DOAB), and the Verzeichnis Lieferbarer Bücher (VLB).



### **Print on Demand and Multiple Formats**

MDPI Books are available for purchase and to read online at any time. Our print-on-demand service offers a sustainable, cost-effective and fast way to publish MDPI Books printed versions.