

Talking points for conversations with caregivers about their loved one with Fragile X Syndrome



1 Discuss the trial process and eligibility to determine if the patient is appropriate for the trial. This includes: trial timeline (including number of in-person and virtual visits), trial objective, and inclusion and exclusion criteria



2 Explain that while the potential effects of the investigational medication are not yet known, one of the main goals of this trial is to study cognition, as prior research shows that improvements in cognition may be closely linked to better daily function and behavior in Fragile X Syndrome⁵



3 Share helpful resources that provide detailed information about the study and what to expect

Visit mirumclinicaltrials.com/fragilexstudy/hcp to learn more about the BLOOM clinical trial.



References: 1. Clinicaltrials.gov. NCT07209462. Updated October 24, 2025. Accessed October 30, 2025. <https://www.clinicaltrials.gov/study/NCT07209462> 2. Data on file. Mirum Pharmaceuticals, Inc. 3. Berry-Kravis EM, Harnett MD, Reines SA, et al. Inhibition of phosphodiesterase-4D in adults with fragile X syndrome: a randomized, placebo-controlled, phase 2 clinical trial. *Nat Med.* 2021;27(5):862-870. doi:10.1038/s41591-021-01321-w 4. Kelley DJ, Davidson RJ, Elliott JL, et al. The Cyclic AMP Cascade Is Altered in the Fragile X Nervous System. *PLoS One.* 2007;2(9):e931. doi:10.1371/journal.pone.0000931 5. Dakopoulos A, Condy E, Smith E, et al. Developmental associations between cognition and adaptive behavior in intellectual and developmental disability. *J Neurodev Disord.* 2024;16(1):31. doi:10.1186/s11689-024-09542-z



For US Healthcare Professionals



A Mirum Clinical Trial for Fragile X Syndrome
Information for Healthcare Professionals

Every inch can seem like a mile

Fragile X Syndrome can make everyday moments feel out of reach. **Together**, we can learn more through research.

Consider referring your patients to our BLOOM clinical trial.

MRM-3379 is an investigational medicine being studied in Fragile X Syndrome¹

Currently, there are no approved treatments for Fragile X Syndrome. The BLOOM clinical trial is studying if MRM-3379 can safely and effectively help improve certain symptoms in male participants with Fragile X Syndrome.

BLOOM is a multicenter, double-blind, randomized, placebo-controlled, phase 2 trial enrolling male patients aged 16-45. In parallel, male patients aged 13-15 will be enrolled in an open-label arm.¹



Primary endpoint¹:
Safety and tolerability



Key secondary endpoint²:
Cognition assessed via National Institutes of Health Toolbox Cognitive Battery (NIH-TCB)

Study design for the Bloom clinical trial²



Trial eligibility includes*:

- ✓ Confirmed Fragile X Syndrome diagnosis
- ✓ Males aged 13 to 45

*Other eligibility criteria may apply.

Dosage¹

Open-label arm: Males 13 to 15 years old

- Low dose

Double-blind arm: Males 16 to 45 years old

- Placebo
- Low dose
- Medium dose
- High dose

Endpoints

Primary endpoint

- Safety and tolerability
 - Incidence and severity of treatment-emergent adverse events
 - Withdrawal due to adverse events

Key secondary endpoint²

- Cognition, measured as change from baseline at Week 12 on the NIH-TCB Crystallized Cognition Composite (Picture Vocabulary Test (PVT) and Oral Reading Recognition Test (ORRT))

MRM-3379 proposed mechanism of action

In Fragile X Syndrome, loss of Fragile X messenger ribonucleoprotein (FMRP) is correlated with increasing disease severity and decreasing cyclic adenosine monophosphate (cAMP) levels. Increasing cAMP levels may help correct the signaling disruption caused by Fragile X Syndrome.^{3,4}

MRM-3379 is an investigational medication designed to block phosphodiesterase-4D (PDE4D) in the brain to increase cAMP levels.³

