Industry: Pharma
- Target Indication: Fragile X syndrome and other autism spectrum disorders
- Future indications: variety of neurological disorders

Management
- XXXXX XXXXX
  Executive Chairman & CEO
- XXXXX XXXXX
  Scientific Founder & Chief Scientist

Advisory Team & Board of Directors
- XXXXX XXXXX
- XXXXX XXXXX

Fragile X KOLs
- XXXX XXXXX
- XXXX XXXXXXX
- XXXX XXXXX

Executive Summary:
- XYZ Biotech is a pre-clinical-stage company taking a targeted approach in the development of small molecule therapeutics to treat fragile X syndrome (FXS)
- XYZ Biotech is utilizing a structure-based design to effectively ‘thread the needle’ in developing selective modulators of key serotonin (5-HT) receptors believed to be involved in FXS and other neurological disorders
- Two distinct, first-in-class drug series have been developed that selectivity modulate unique combinations of receptor subtypes—with minimal off-target receptor binding that can lead to side effects seen with other drugs from this class
- XYZ Biotech is positioned to deliver two first-in-class drug candidates for clinical development approximately 12 months after program funding, and their intention is to pursue orphan drug status for both programs
- There is potential to pursue multiple indications beyond FXS, including other autism spectrum disorders, binge eating, schizophrenia, mania and addiction with these two series

Market Opportunity/Unmet Need:
- FXS is the most common monogenic autism spectrum disorder (ASD)
- Typically diagnosed in early childhood, at 2 to 3 years, based on symptoms and confirmed by genetic analysis - well after significant neuronal impairment has occurred
- Beyond core ASD symptoms, such as repetitive stereotypical behaviors and deficits in social functioning, cognitive impairment and anxiety often occur
- Hyperactivity, attention deficit, psychosis/mania, hypersensitivity to sensory stimuli, and/or increased seizure potential may also be evident
- No currently approved drugs for treating the core symptoms of FXS
- Depending on the patient, anti-anxiety agents or serotonin selective reuptake inhibitors (SSRIs) can mitigate some of the behaviors that accompany FXS; however, limited efficacy may be achieved and side effects are a frequent issue
- Buspirone is used off-label to treat the repetitive behaviors and anxiety resulting from FXS, but it causes sedation and it brings cardio-tox risk

XYZ Biotech Pipeline:
- ABC-001 has a unique profile with 5-HT1A, 5-HT2C, and 5-HT7 partial receptor agonist activity
- Lead compound, ABC-001, is highly effective in decreasing repetitive behaviors and motor stereotypy, and increasing social functioning in mouse models, suggesting efficacy in treating core fragile X symptoms
- Selective activation of target receptors, with minimal effects at other receptors, represents a unique receptor modulation profile
- XYZ Biotech anticipates that ABC-001 will have minimal side effects, such as suppression of locomotor activity, sedative/stimulant activity, or cognitive impairment seen with other drugs
- ABC-001 achieves efficacy similar to Buspirone in mouse models of repetitive behaviors, anxiety, social and cognitive deficit—but without sedation or the cardiovascular toxicity risk
- ABC-002 exhibits a different, complementary, pharmacological profile with the potential to address cognitive dysfunction, attention deficit, hyperactivity, and psychosis associated with FXS and other autism disorders by selectively enhancing 5-HT2C signaling while reducing 5-HT2A/2B signaling
- Lead compound, ABC-002, exhibits a first-in-class pharmacological profile
- XYZ Biotech anticipates that ABC-002 will not produce the sedation or weight gain that typically accompany other antipsychotic drugs frequently used in FXS patients

Technical Milestones Achieved:
- Preclinical evaluation of the ABC-001 and ABC-002 series has confirmed that both platforms have favorable pharmacological profiles
- Both are efficacious with oral dosing, selectively modulate serotonin receptor activities with minimal off-target effects, demonstrate therapeutic efficacy and safety in animal models
- XYZ Biotech’ compounds have been administered to Rhesus monkeys and demonstrated behavioral efficacy at 10 mg/kg and did not cause adverse effects such as nausea, sedation, movement disorders, or anxiety-like behaviors
- Extensive PK/metabolic profiling data with no toxicity observed in preclinical models
- Necessary chemistry is in place for scale-up to support advanced studies of both candidates

Intellectual Property
- Exclusive worldwide license to all technology
- Several issued patents covering composition of matter, methods of treatment, novel bio distribution through 2028
- Pending patents could extend IP coverage through 2035

Non-Dilutive Funding to Date
- $10M in NIH & DOD grants

Seeking a $20m Series A Round
XYZ Biotech anticipates achievement of the following milestones post financing
- File (12 months)
- Complete Phase I studies of both compounds (24 Months)
- Complete Phase IIa,b trials of both compounds (36 months)