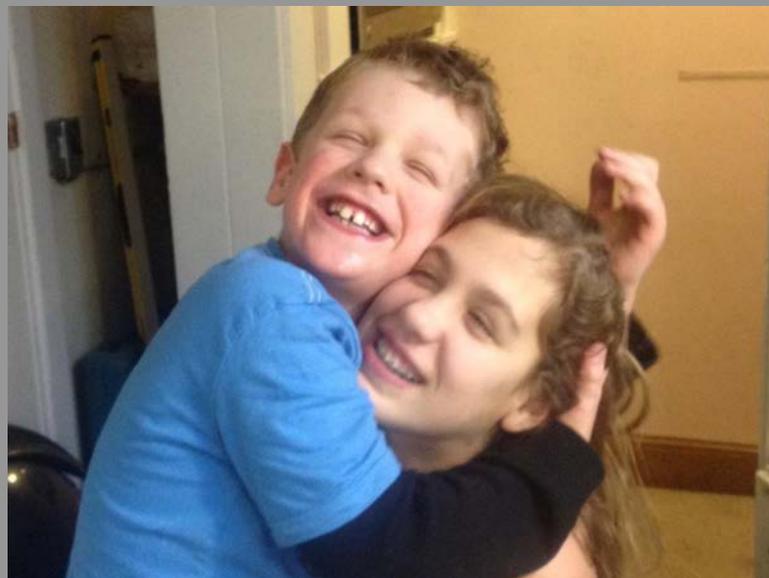


A Plea for Help
from
Fragile X Families



Fragile X Syndrome Facts

Fragile X syndrome (FXS) is the most common known cause of inherited intellectual disability. Symptoms of FXS include a range from learning disabilities to more severe cognitive or intellectual disabilities that were previously referred to as “mental retardation.” Delays in speech and language development are common, as are a variety of physical and behavioral characteristics. FXS is also the most common known cause of autism or autism spectrum disorders. FXS is caused by a “full mutation” of the FMR1 Gene.

Features of Fragile X Syndrome in Males

- The majority of males with fragile X syndrome demonstrate significant intellectual disability. Disabilities in FXS include a range from moderate learning disabilities to more severe intellectual disabilities.
- Physical features may include large ears, long face, soft skin and large testicles (called “macroorchidism”) in post-pubertal males. Connective tissue problems may include ear infections, flat feet, high arched palate, double-jointed fingers and hyper-flexible joints.
- Behavioral characteristics can include ADD, ADHD, autism and autistic behaviors, social anxiety, hand-biting and/or flapping, poor eye contact, sensory disorders and increased risk for aggression.
- No one individual will have all the features of FXS, and some features, such as a long face and macroorchidism, are more common after puberty.

Features of Fragile X Syndrome in Females

- The characteristics seen in males can also be seen in females, though females often have milder intellectual disability and a milder presentation of the syndrome’s behavioral and physical features.
- About one-third of females with FXS have a significant intellectual disability.
- Others may have moderate or mild learning disabilities, emotional/mental health issues, general anxiety and/or social anxiety.
- A small percentage of females who have the full mutation of the FMR1 Gene that causes FXS will have no apparent signs of the condition—intellectual, behavioral or physical. These females are often identified only after another family member has been diagnosed.

Source: The National Fragile X Foundation

Ayden AbouElSeoud

Age 5

Holt, MI

By Courtney AbouElSeoud

My son Ayden turned five last summer and, in the fall of 2012, he started kindergarten. We quickly saw that he had many troubles focusing and lots of anxiety in the classroom setting that was preventing him from learning as well as we knew he could. We started looking into solutions from other families with the same genetic disorder that he has and were repeatedly pointed to one specific drug that was in late clinical trials. In October of 2012 we enrolled him in a Phase III clinical trial for a drug called Arbaclofen (STX 209) which is being researched by Seaside Therapeutics Inc. Ayden has fragile x syndrome, and this drug is specifically targeted towards individuals with fragile x. While the trial itself was targeted toward social function, we knew that many others who had been in the same drug trial were seeing other benefits of the drug including increase in speech. We went in hopeful, but hesitant. Our son had around five spoken words at the time. To say he had them is misleading though, as to get him to use them was an effort in itself.

The trial itself was a double blind trial. We had a chance to get the placebo. There was also a chance it wouldn't work or that he would end up on too high of a dose. The silver lining for us was that when we finished the Phase III trial we would be allowed to roll into an open label extension where we knew he was getting the actual drug and could control the dosage (to a certain extent). We almost immediately saw results in Ayden upon starting the blind trial. Within a week he was going places he would have had to be carried into kicking and screaming. Within a month he had started to use words spontaneously. By the end of the third month, we had seen improvements in every area of development. These results were mimicked at school where we saw increases in every single area, socialization the most. Ayden is in an autism class at school where they runs tons of data so we knew exactly how those improvements were showing.

As of today, we are on our seventh month of Arbaclofen. Ayden's development over the past months has surpassed what we ever could have hoped for. He is speaking in small sentences. He can isolate every sound in the alphabet. He can trace all his upper and lowercase letters, and according to his testing (which was modified because he doesn't speak) is at his grade level enough where he will actually move into first grade, on par with his peers curriculum wise. He more than surpassed every single IEP goal this year, and many of those were set high as we want him to be challenged.

On May 15, we received news that shook our fragile x community, the open label extension for Arbaclofen



was to be terminated immediately. We would receive information from our clinic on how to wean our children off the medication and that process would begin no later than May 31.

I've often heard fragile x described as having the sound of a vacuum in your head. Consider going through life daily with that sound and attempting to speak or to walk into large rooms full of people. Imagine how difficult it would be and how easily you could become frustrated. Imagine then that there was something you could do to shut that noise off. That is how I look at Arbaclofen - as an off switch for that constant noise in Ayden's head. Now we have to turn that switch back on. Devastated does not even begin to describe the emotions I am feeling. Terrified. Worried. Overwhelmed. Sadness. Anger. And there are tears, lots and lots of tears.

My son went from barely speaking, struggling socially, with anxiety issues, and a lack of self-awareness to a little boy who was making friends, playing with those friends, playing with toys correctly, jumping into new situations with ease, going into loud places, laughing, talking. TALKING.

We were told the open label would be available until FDA approval (or lack thereof). We were told we would have 4-6 months' notice if they were to ever stop the open label. And instead we have a few weeks, all because of funding. That this comes down to funding when we are so close to seeing it approved is just not fair. We are looking at the possibility of not only losing the medication, but losing the advances that have been made as well. I am facing a reality where I may never again hear my son say "mommy".

I am writing this, sharing our story with you and hoping. We need someone to help. To help Seaside finish clearing the FDA hurdles to get this drug on the market. To help with much needed funding to keep this promising drug on track and eventually getting it into the hands of those individuals, like my son, who need it the most.



Kolton Acree

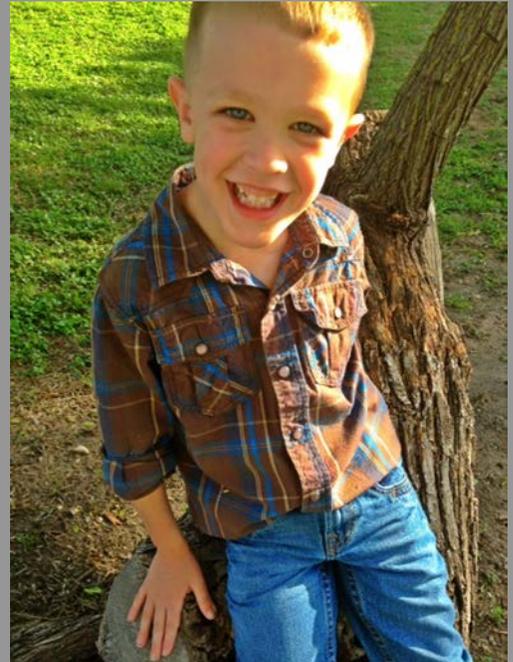
Age 5

San Antonio, TX

By Deanna Acree

I am writing this in a plea to help all the families I love, families I am friends with and have dinner with and families I have never met beyond an email, families I know just from Facebook. I may have never met many of these families, but we are joined together by love, hope, and passion for our children whom have been diagnosed with Fragile X Syndrome. Fragile what? Yes we get that a lot! Fragile X is a genetic cause of intellectual disability, leading known cause of Autism, what you will never read when you research Fragile X, is how amazing a person with Fragile X is! I should know because my 5 year old son was diagnosed three years ago. When we received our diagnosis, I had little hope, doctors were grim on what they believed he would do, could do or should do in his lifetime. Soon after I enrolled my son into a research study for language development and for the 1st time felt like my son and I belonged, we were understood, we were welcomed, and we were special! That very short research study changed my whole outlook, gave me hope, and introduced me to families that have become my best friends. As a mother, I am the voice of my son, I am his advocate. He needs me! He also needs others, doctors who believe in his potential and research studies that believe that one day a cure will be found.

Recently, STX209, a study targeted for Fragile X and Autism has announced they will be closing the study due to lack of funding. This is beyond devastating, beyond heart breaking, it simply cannot be! My son was not a participant of this study, but so many children with Fragile X have taken part and have a new life because of this study medication. I have watched with my own eyes the transformation these children have made. It can only be compared to a caterpillar turning into a butterfly! Imagine your child is unable to tell you their tummy hurts, unable to make friends at school even though they love other kids, unable to run a quick errand with you because the store may overstimulate them, unable to look at night while you tuck them into bed. Just Imagine! Your child is locked in world that no one can understand, they try and try but everyday life can be a struggle. A simple change in their routine can upset their whole day. This is what life has been like for many families I know before STX209. Now imagine this, your child is sick, he can tell you what hurts, he goes to school and has friends high fiving him in the hallway, he can and wants to go to the mall, he hugs you before bedtime and whispers I Love You. He is living in a world that he deserves to be a part of, all because of this new medication that soon could not be available! How do you look at your son or daughter and tell them their time is this world is over, it's time for them to go back to the world they once lived in, scared, anxious and overstimulated? You don't, I will not sit here and believe that this is can end; I know life is not fair but this is beyond unfair. This is not acceptable! STX209 needs funding now, today, as part of the Fragile X community, I will not sit back and see what happens. Fragile X children are incredible, they change your life in more ways that can be counted. It should be on everyone's bucket list to meet and fall in love with a child that has Fragile X! Please help us in our fight to keep research study STX209 open! This is our future, their future, my future, and my son's future.



Ian Carver

Age 11

Henrico, VA

By Kristin Dochterman Carver

My son is 11 years old. He is in fifth grade and will be attending middle school next year. He has a great sense of humor and loves Sponge Bob and PBS Kids. He also has great anxiety in new situations and struggles with counting to 20. Fragile X Syndrome affects his life, and ours, in so many ways. It has become our “normal” but about six months ago we were given a chance to try for a new normal.

A few days ago, that chance was taken away.

We heard about STX209 through our fellow FX parents on Facebook. Their children were seeing positive changes. The results varied, but they all appeared to be good. We decided to investigate further. We tried to get in on a study at one site, but it fell through. Then another opportunity came up at Duke University, and we got in! We had our first visit on February 5. It was a long and stressful day. My son is a textbook case for social withdrawal, and I knew we would have no trouble getting him into the study. We didn't. We were excited for our next visit on March 5 when he would begin the dosing.

And then things changed.

We received a call that said we may not be eligible to receive the drug after our participation in the study was complete. It wasn't certain either way. We decided to go ahead and take a chance.

We will never know for sure whether Ian was on a placebo or the medication itself, but I believe he was receiving at least a low dose. During his time receiving the dosage, I started to see the child that I know he can be. He was more relaxed. He spoke more, especially at school. His teacher and speech therapist noticed positive changes in behavior and speech. He was happier, and isn't that what every parent wants most for their child?

Right before our last visit to Duke, we received word that Ian was definitely not going to receive the medicine after our participation was done. We were naturally disappointed but not surprised. I took solace in the thought that the trials were wrapping up and the push for FDA approval would be the next step.

And now, we don't even know if that is going to happen. And my heart is breaking for the other families who have been doing this for years and are now having it taken away.

So, we will go back to our normal and pray that somehow, some way, we can find a way to get STX209 to the children who would benefit from it so much.



Andrew & Jason Frech

Age 9 & 7

Berwyn, PA

By Kara M. Frech

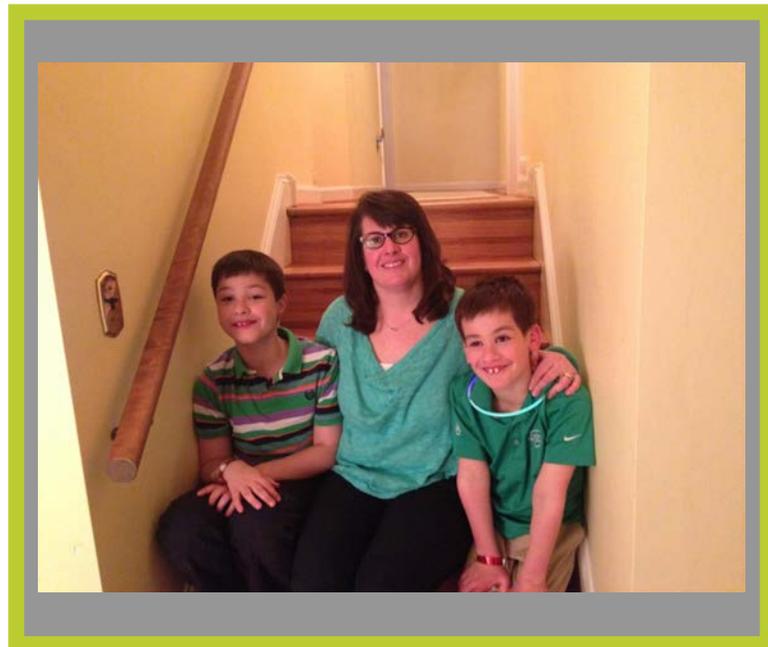
Both of my sons, Andrew (9) and Jason (7), have been participating in a clinical trial for STX209. Andrew has been involved since the Fall of 2009. We have seen an extremely positive difference in verbal skills, social skills, agitation and cognitive ability in him. Before the trial, Andrew would want to play with other kids and would grab at them from the peripheral. Now he asks to join in on the games with the other boys at recess and gets invited over for play dates. This kid, who wanted to buy lunch at school like the other kids but did not know he needed to use his words to ask, now requests to have "pokemon cards" because he wants to trade with his friends. This is the same child who would once fall to the floor and tantrum at us at the word "no", now comes back at us and asks "Why not?" like any typical child. A child for whom speaking was an issue just gave a 7 minute oral biography in front of his class. His reading has progressed to a 2nd grade level. Math while still an issue is doable and progressing.

Jason has been involved since Fall of 2011 due to age restrictions. For him, the effects have been in the social, verbal and cognitive areas. He is more interested in what his peers are doing. He wants to compete in all sports. He plays interactive games and verbally jokes with us. He tells us what he wants or dislikes. If he has an ear infection he is finally able to communicate this to us, not go on for a week with miserable behaviors. He is able to get up and perform in class shows, where in the past he would have had a tantrum and taken off his clothes.

While these are all positives, this medication is not to be confused as a cure, it simply makes the quality of life for my boys a whole lot better. They are much less defensive about the world around them. It enables them to be an active participant in it.

I fear much regression at the discontinuation of the trial. There are currently no medications on the market for Fragile X Syndrome. I ask your help in funding the extension so that we do not see the regression and loss of skills.

Thank you for your time.



Nathan & Zakira Freedman

Age 7 & 12

Newton, MA

By Aimee & Josh Freedman

How do we even begin to say what STX209 has done for our family, what it has done for our son, our hope for what it could do for our daughter?

Parents of children with special needs are often told that their feelings of grief are similar to the feelings of a death of a loved one. You need to mourn the life that you had envisioned for your child and your family and start a whole new dream, or reality. We had already said our goodbyes to the life we thought we were setting ourselves and our kids up for, we were moving on and looking to the future with hesitation and a good dose of fear. When we found out that we could participate in a drug trial for our son, a drug that was already in Phase III, we really went after that opportunity and we were so excited for the chance to help get something going for all of the families that are affected by Fragile X Syndrome. After 20 months of watching Nathan progress incredibly, we are now told it will all be taken away – sorry folks no more money for your child to keep taking a medication that has been absolutely life changing. Now we must mourn again, once simply wasn't heartbreaking enough. Now that we have seen our child happy, learning how to read, talking so much, participating in ways we had only imagined were possible, having haircuts that we don't have to hold him down for, waking up happy instead of screaming – now that we have seen it we will have to watch it all be taken away. We have been watching, listening and taking in all that he is doing as the gift that it is because it is now so close to being taken away from us and from him. How do we watch our child regress? How can anyone watch their child regress into their world that is so hard and uncomfortable for them that all they can do is yell?

We have to believe that all this research is to a point, all the blood work was to a point, all the paperwork and schlepping – all of it has to be for a point. There has to be a solution to this. So many families have seen wonderful results on the STX209 and all of their worlds are being turned upside down as we all try to figure out what to do next. Who will help our children, our families? So much time, money, blood, sweat and tears have been invested. This medicine can't just go away. When your child has a genetic diagnosis that makes life so challenging for them, one of your only hopes is medication and research. Families are waiting for STX209 to be approved by the FDA so they can give it to their kids. We are waiting for it to be approved so we can give it to Zakira, Nathan's older sister.

Thank you for your time.



Grady Friedman

Age 2

Flagstaff, AZ

By Christie Clark Friedman

After receiving our son Grady's diagnosis on his 18-month birthday a mere 13-months ago, we were devastated and fearful about what his future would bring. We were consumed by the fear and uncertainty about what his life would be like. Would he ever speak? Would he have friends? Would he ever be able to work and live independently? The list of fears goes on and on. We never expected to receive such life-changing news. We quickly found ourselves connected with other families like ours; almost immediately we began to feel hope. We began to feel hope because we learned about the current direction of Fragile X research, and the number of clinical trials available to affected individuals. We heard the before-and-after stories of many families; the results were inspiring, and life altering to say the least.

Our son is only 2-years old, so he is unable to participate in drug trials at this point. I know that I speak for our whole family when I say that we are so thankful to those families that have made the decision to participate in clinical trials. We know this process is not easy for them, as it often includes weaning off all other medication for a period of time before starting the clinical trial. This period of time is quite difficult for most families, yet they trudge on.

The STX209 study using the drug arbaclofen has been a miracle drug for many, many families. Children who didn't speak, now have increased vocabularies and are able to communicate wants and needs. Children who wouldn't walk into an unfamiliar environment without melting down for hours are now able to do so with little to no difficulty. Children who suffered from violent and unpredictable outbursts now have better impulse control and no longer lash out uncontrollably. For us, seeing these results has instilled so much hope for Grady's future.

To see a drug that so clearly works get discontinued for lack of funding is absolutely heartbreaking. While we know that these studies require tens of millions of dollars to maintain, to be so close to obtaining that higher quality of life for our children to have it stripped away is barbaric. How are we as a community supposed to go back to a lower quality of life when we know a better one exists? Arbaclofen may indeed be the key that allows Grady access to the quality of life that all parents dream of for their children. Like many in the Fragile X community, we feel like a great measure of hope has been stolen from us.



Logan Kisamore

Age 9

Baton Rouge, LA

By Patricia Kisamore

This picture is brought to you by Stx-209. A little boy playing a video game with his friend, not to unusual right? It is if that little boy has Fragile X Syndrome and this was his very first birthday party, at 9 years old.

Getting the diagnosis that your child has a disability is crushing. You hear words like mental impairment, cognitive delays, social withdrawals, and language delay and your world feels like its spinning out of control. You grieve and grieve and then realize you cant just sit there so you find a glimmer of hope in the form of an experimental drug. You grab it and hold on for dear life. You take your child out of school and drive round trip 675 miles both ways every 6 weeks for just a chance. You subject him to blood draws, psychological testing, and all kinds of discomfort and look him in the eyes and tell him the why: to give him a better life. Then, you hit the lottery and get it and you see progress, real progress. You get raving reports from school, kids stop you in the hallway to tell you about your son, you see him pretending to be Batman and Superman and The Rock. You hear him use more words and his grandparents hear PawPaw and Nana. And then you hear the company has run out of money, thats it, no more.

This little boy gets it! He has never willing taken medication but he knows this little white pill that he asks for, has opened up his world. My heartbreaks at the thought of having it end. This time I have to look into his eyes and say "I am sorry, no more pills" and hope and desperately pray that he doesn't go back into that bubble. We were so close but now we need a hero, or two, to help us help these kids have the life they deserve. My son adores super heroes, and we need one now more than ever. Let me look him in the eyes this time and say "There really are superheroes out there".



Wyatt Lungarini

Age 4

Ansonia, CT

By David & Sharri Lungarini

We are parents of a special little 4 ½ year old boy named Wyatt. He began receiving intervention services in 2009. The level of services and the amount of people we worked with increased dramatically in February of 2011 when Wyatt was diagnosed as being on the Autism Spectrum. Soon after, he was also diagnosed with the genetic disorder Fragile X Syndrome, which is the #1 known genetic cause of Autism.

Our family and our home became hosts to a special group of Physical Therapists, Psychologists, Speech Therapists, Occupational Therapists, Developmental Therapists, and Board Certified Behavioral Analysts five days a week all through 2011. It goes without saying that these services ramped up at what was a very painful and difficult time for our family. Wyatt was totally withdrawn into himself and every day we ached at how much of a struggle his life had become. The one light piercing the darkness was the incredible team of professionals who worked tirelessly with him and with us. The Birth to Three Team was as helpful during our dealings with the School System as we prepared for Wyatt to turn 3 years old. Thanks to their tireless efforts we had an incredible transition from Birth to Three to the ACES school in North Haven Connecticut where Wyatt has been for over a year.

Despite these years of hard work for hours a day, 7 days a week, the honest assessment is that while Wyatt has made much tangible progress he is still essentially a toddler developmentally, non-verbal, filled with anxiety, and largely trapped inside of his own head. Therapeutic and teaching approaches have allowed Wyatt to make advances but they are small and incremental. The stark reality is that there is little to no chance that Wyatt will ever be able to live on his own, be mainstreamed in school, or enjoy the thousands of things we all take for granted in life.

We have followed with great interest a drug trial that has been ongoing through Seaside Therapeutics for STX209. The study has been in progress for over 3 years and we know many people with children who are included in the trial. We have heard the success stories and have seen the transformative effects. Children who were riddled with anxiety, trapped in their own minds have come out of their shells. They have made friends, laughed, played, lived. Parents have heard "I love you" for the first time or have gotten their first hugs or have been able to go to a restaurant for the first time in years. We know that there is no miracle drug, and that everyone reacts differently to medicine. However, we were filled with hope that this medicine combined with our current school and home programs would represent a potential for a much more fulfilling life for our son. We knew that STX209 was in open label testing and were very hopeful that FDA approval would be available within the next couple of years. However, when we heard that the trial was suddenly ended, so close to the finish line, we were devastated. This drug could impact thousands of lives, and the end of the trail forces children already taking the drug back into the Fragile X box and it prevents a great opportunity to ever let Wyatt out of it. We love our son, we need this trial to continue, for this hard work and progress to have not been made in vain, please help us shine a light in the darkness. Thank you.



Caleb Welin

Age 9

Cambridge, MA

By Melissa Welin

In April of 2006, my son was diagnosed with Fragile X Syndrome at the age of 22 months. Caleb is an amazing boy who is full of life and love but he struggles to share that with the world sometimes because of the effects of fragile X.

For the last seven years my husband and I have been waiting for the cure we were told that we would have in our lifetime. Last fall we took steps to participate in a drug study for STX 209, a drug being tested by Seaside Therapeutics, Inc. to treat the core symptoms of fragile X.

We were uncertain about participating in the trial initially but we heard such promising stories from other members of the fragile X community that we decided to take the chance. My husband drove Caleb to the study location many times over the course of the trial, sometimes we had to take Caleb out of school early for the visits. We decided some missed school was less important than what we could gain from taking part.

When we completed the double blind placebo controlled Phase III study we enrolled in the open label extension with no second thoughts. We have seen amazing progress for Caleb since that time. His language has improved, he is more social, he is able to do things, like go to the circus that he could never do previously.

And then I learned that the extended trial of STX 209 is over. We had heard rumors of money troubles but we all hoped for a solution and some advance notice. We didn't get either.

I woke up the next morning and stood crying at the counter while making Caleb's breakfast, which is always accompanied by a spoon of applesauce and his morning meds, knowing that we were one dose closer to the end.

I'm not even sure where I should to begin. I'm wracking my brain trying to find the answer. I know nothing in this business is guaranteed, there's a lot of money pharmaceuticals because it's expensive work. Changing the world takes lots and lots of money.

I refuse to believe that this is as far as the story goes. I've never expected a fairy tale ending, but this defies logic. We are *almost* there. In the face of incredible odds, Seaside has gotten us to the point where we can see the goal line. Now we need that one person to stand up and either clear the way or give them a nice big *push* from behind.

One person, that's it. Please help me find that person.



David & Jonathan Wright

Age 13 & 6

Visalia, CA

By Tiffany Pace

As my oldest son, David's, 14th birthday approaches in just a couple of weeks, I've been reflecting back on the past 14 years of life with David and the journey we have taken as a family in all of that time. David was diagnosed with Fragile X syndrome on September 16, 2002 and the roller coaster called life definitely picked up speed and added a lot more twists and turns and flips. Since David was born, we added Nathan in February 2002 and Jonathan in February 2007. Nathan is not affected by Fragile X but Jonathan, like David, is affected.

We have tried many drugs over the years to try to treat the symptoms of Fragile X for David and then for Jonathan too. When I learned that David qualified for the STX209 (Arbaclofen) clinical trial I was ready to jump right in with both feet. We had some delays in getting him started due to his age at the time. We just had to wait for them to be ready for trials with the next age group younger than what they were doing at that time. Finally it was our turn and we began our journey with Arbaclofen. David and I drove to the MIND Institute at UC Davis in Sacramento every 2 weeks in the beginning. We drove 4 hours each way, usually going up the night before an appointment and staying at Ronald McDonald House or at a nearby hotel because I couldn't handle the drive both ways on my own in one day. David couldn't either to be honest. Each time a blood draw was required at a visit, we had to strap David to a board on the floor and then several people, myself included had to still hold him down and still in order for the phlebotomist to be able to draw his blood. I suffered countless bruises from him biting me, hitting me, pinching me, kicking me during these times of lab work, but that wasn't the only time I had bruises from David doing those things to me. He had been biting me since he got his first tooth at 4 months old. I regularly had bruises on my arms and around my collarbone from his bites.

At the time we began this trial, David had also begun starting to have episodes of rage where he would throw anything near him, yell as loud as he could, scream, cry, hit walls, hit anyone who got close to him, and we were terrified by these rages. During the double blind, he ended up on the placebo for the first part but we didn't know that for sure until we had moved on to the extended, open label, part of the trial. The second part of the blind trial he had Arbaclofen but I didn't see any dramatic results with him. I was actually hesitant to go into the open label because I hadn't seen any significant improvements but in the open label he was able to have a little bit higher dose than before and we knew he was receiving the real medication every day. After a bit of time on the steady dose of Arbaclofen, I noticed he did better in the car,



not getting anxious as much when we drove long distances. I noticed his rages went away. I noticed David saying, "I love you" to me without me saying it first, without anyone telling him what to say. He was just telling me how he feels about me! I have heard many things I never thought I'd hear from David in the last 3 ½ years. I love you, I miss you, I'm tired, Can I take a shower?, Is it time for my medicine?, Can I have [insert just about anything here]?, I'm frustrated, I'm confused, I'm embarrassed, I don't like that, I like that, It hurts, etc. I've also seen so many amazing things in the time since David started Arbaclofen. He started reading! Not just sight words that he'd memorized but really READING! We never thought he'd learn to read at all. He started doing math problems on his own, addition, subtraction, multiplication, division! His handwriting became legible! He began participating in class/group activities. A year ago, we were planning his very first, EVER, birthday party, WITH FRIENDS! He told me who he wanted to invite, I invited them, they came, they all had a good time, even David and he was so happy! He has let us sing "Happy Birthday" to him for his last 2 birthdays and that was never possible before Arbaclofen. One of the biggest changes I've seen in David in the last 3 ½ years though is that he now freely gives out hugs and kisses to me and his grandparents and others he loves and cares about. When he was little I couldn't even get him to hold my hand when crossing a street. That kind of small touch caused a complete meltdown for him. He hated being held as a baby and that didn't improve until he got Arbaclofen and suddenly he wants to hug me all the time and he wants to kiss me goodnight and he wants me to sing to him at bedtime. He wants to hold my hand when we are walking together in a store or parking lot and he smiles so much that my heart aches at the thought of that smile going away with this drug that has put more hope into our lives than we ever imagined.

Losing this drug is so terrifying to me. I am a single mother and I am terrified. What if his rages come back? What if he stops reading and doing math? What if he stops enjoying his social activities at school and church and with our family? What if the young man I've gotten to know in these past 3 ½ years goes back into hiding? I'm scared for David. He KNOWS he is different and he knows when he is doing something that he can't control and that other people are noticing that. He hates knowing that he is different. He just wants to be a 14 year old kid like the rest of his friends. He wants to play basketball and go to his classes at his middle school and give high fives to his friends and teachers. He wants to keep moving forward, just as I want him to keep moving forward. I'm completely devastated at the idea that he might slide backwards to a place where no one can reach him again. I hope that he will have matured enough in these past few years to keep some of the progress he's made but I don't know. All I know is that this drug that has changed our world, changed David's world is being taken away from us and so abruptly that I don't know how to even explain to David what is going to happen, even if I knew what was going to happen.

Jonathan has been on Arbaclofen for almost a year now and we have only just started to see his potential emerging with this drug but I hate to take away the possibilities from him like this. I hate that I have no choice, no control, no say in the matter. Both David and Jonathan play baseball in our local Miracle League and while baseball is not their sport of choice, they love playing together and they love spending that time with other kids and with me and Nathan and enjoying being outside, running the bases, listening to the crowds cheer and yell their names. They have thrived and I am terrified that they will cease to thrive when Arbaclofen is taken away from them.



Matt Zolecki

Age 12

Plainfield, IL

By Missy Zolecki

We entered into the Phase II STX209 trial in August 2009. After completing the 2 portions of that trial, we were offered an opportunity to begin an open label extension. December 2009, Matt began that medication. We had been through the gamut of medications prior to starting this trial, without much success at controlling his challenging behaviors. He had gained a tremendous amount of weight as a result of the side effects from the medicine. He had significantly regressed in school as well.

As you can see the physical difference is amazing. He was able to discontinue the medication that was causing the weight gain. He made many other changes. In 2009 it took 5 people to hold him down to get his teeth cleaned at a specially trained pediatric dentist office. He now goes to our family dentist, completes his visit while I remain in the waiting room and doesn't require any holding! He has become interested in running and has now competed in the Special Olympics, run a 2K and 2 5k's!!!

We have traveled to one of our favorite places, Disney World, multiple times. It wasn't until last summer that he showed interest in riding some of the more intense rides. He was so proud of himself after going on those rides, as were we! We have seen his ability to verbally communicate grow. Increased language, with also an increase in conversational speech. We have seen him have imaginative, interactive play! Although that may not sound like a big deal, but for years we have bought him gifts hoping he would play with them at least partially appropriately. Finally, he is!!

Birthdays should be a joyous time, but for Matt they have always been overwhelming! Each year since starting this medicine we have been able to watch him have his entire lunch room of peers sing to him! We had not sung Happy Birthday for years, as he would have an intense meltdown as a result. This past year he requested a birthday party. He told us he wanted to go bowling, and even made his sister write a list of all the guests he would like to invite! He thoroughly enjoyed his day, as did we!

Our quality of life has changed in a way we never anticipated! We are very nervous about what the future holds as we wean off of this medication. We hope we don't return to where we were in 2009.

