



INCLUDE

(INvestigation of Co-occurring conditions across the Lifespan to Understand Down syndromE)

Michelle Pfeiffer: Researching Unanswered Questions for Children with Down syndrome

When Michelle Pfeiffer's daughter Anna was just 15 months old, she was diagnosed with Acute Myeloid Leukemia (AML). Pfeiffer then was told something she had not often been told by doctors: "You're lucky Anna has Down syndrome."

While children born with Down syndrome diagnosed with AML often fare better than children who do not have Down syndrome, for other health conditions and problems they may fare worse. Why? Children with Down syndrome face many of the same challenges and health problems as other children, but too often they are left out of clinical research.

We do not always have answers to these very important questions. Anna is a healthy and happy teenager today who rarely gets sick. But her early bout with leukemia led Pfeiffer down a path of wanting to help answer these unanswered questions. Pfeiffer is the founder and director of Anna's Angels, a foundation that funds and promotes research that improves the cognitive and communicative abilities of people with Down syndrome.

Children with Down syndrome are often not part of therapeutic research for many reasons, including an unwillingness by sponsors to include them, a lack of funding, and barriers to participation. The INCLUDE project (INvestigation of Co-occurring conditions across the Lifespan to Understand Down syndromE) is an effort by the National Institutes for Health to better fund and promote research that improves health and quality of life needs for people with Down syndrome.

The Pediatric Trials Network is partnering with INCLUDE to develop clinical trials and research opportunities to test and better understand therapeutics in children with Down syndrome.



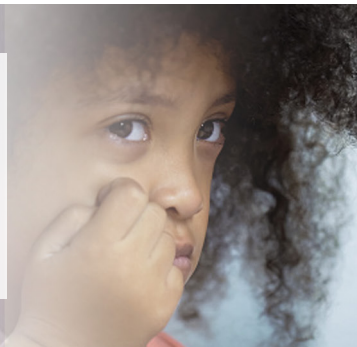
Anna (left) with her brother Andrew and sister Ashley.



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Pfeiffer remembers feeling uncertain about giving Anna over-the-counter medicines when she was a child because there was no research about drug dosing available that included children with Down syndrome. Anna, like many children with Down syndrome, weighed less than other kids her age making weight and age-based recommendations tricky. For Pfeiffer, the research questions she is most interested in are about speech and cognition.

“The more families like mine that are included in research, the more it helps people with Down syndrome. If children are included, we can get better information that will lead to better therapies and better outcomes.” Michelle Pfeiffer

Four years ago, Anna participated in a clinical trial for a drug to improve cognitive ability. Pfeiffer said that the research team did everything right to thoroughly explain the risks and potential benefits to her family and make the experience as safe as possible. But after she and Anna invested a significant amount of time in visits, procedures, and filling out a regular diary, the study was closed because of low enrollment and lack of efficacy. They were disappointed. Since then there have been very few opportunities for Anna to participate in research.

“In the state we have been in, funding has been so small. We need to ask what can we do to help the most number of families and try to focus on what will help the whole population.

If there are drugs available, can we help get them to people with Down syndrome safely and quickly?” she said. Iron deficiency, autism, epilepsy, attention deficit disorder, and sleep apnea are all examples of conditions that affect children’s cognitive abilities. For children with Down syndrome, having these conditions on top of living with Down syndrome makes learning and development even harder. Children with Down syndrome need to be included in therapeutic research in these areas and their special needs considered.

“The more families like mine that are included in research, the more it helps people with Down syndrome. If children are included, we can get better information that will lead to better therapies and better outcomes,” she said.

Pfeiffer is currently the president of GiGi’s Playhouse Raleigh, where all ages are welcome, from pre-natal to adults. The positive and uplifting environment of GiGi’s Playhouse Raleigh empowers those with Down syndrome and their families to reach their highest potential. GiGi’s Playhouse’s custom, research-based curriculum works towards advancing literacy, math skills, gross and fine motor skills, improving low muscle tone, building self-esteem, preparing for the workforce and more while fostering acceptance, awareness and networking resources for parents, siblings and the community. All programs are FREE of charge.

Learn more: <https://gigisplayhouse.org/>



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