

Fenfluramine slows seizure activity in patients

Le Bonheur Children's neurologists recently began enrolling patients in a new clinical trial of fenfluramine, which aims to help minimize the frequency of seizures for children with Dravet Syndrome.

The trial is in its second phase, and Le Bonheur is one of only four children's hospitals in the region to offer the fenfluramine drug trial.

Early results have found that patients who use fenfluramine have had fewer seizures, says Tracee Ridley-Pryor, MSN, RN, CCRC, the Neuroscience Institute's lead clinical research coordinator. Patients on the medication also have shown attention, behavior, learning comprehension and language development improvements.

"The efficacy of fenfluramine has been demonstrated in the number of seizure-free days our patients have



Maelee Peters-Larson, 3, Tulsa, Okla.

experienced while on the investigational medication, up to 29 and 31 seizure-free days, respectively," Ridley-Pryor said. "For these families, this has been the greatest period of time their child has gone without a seizure since receiving a diagnosis of epilepsy."

Kevin and Katie Peters-Larson's 3-year-old daughter, Maelee, is one of the children enrolled in the fenfluramine drug trial.

Maelee had her first seizure at

Fenfluramine trial at a glance:

- Phase two trial
- Treatment for seizures associated with Dravet Syndrome
- Early results show fewer seizures
- Shown to improve attention, behavior, learning comprehension and language development

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Le Bonheur offers VNS for children ages 4 and older FDA approves device for younger ages

Children as young as age 4 are now eligible for vagus nerve stimulation (VNS) therapy, according to new guidelines released by the Food and Drug Administration (FDA) this summer. Previous guidelines approved the device for children ages 12 and older.

Neuroscience Institute Co-director James Wheless, MD, has been part of extensive research examining the safety and effectiveness of VNS devices in young children in the last 22 years. It is estimated that one out of every three VNS devices is implanted in children and adolescents younger than 21 years old. This accumulated experience, along with recent studies, led to the formal FDA approval for children. Recent work found that 48 percent of young children had a reduction in seizure duration; 42 percent had a reduction in severity; and 40 percent showed a reduction in post-ictal severity.



Oliver Prats, 10, Baton Rouge, La.

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4 months old, and her seizures increased in frequency as she aged. At her worst period, Peters-Larson said her daughter would suffer from hundreds of seizures a day, including tonic-clonic, myoclonic and grand mal seizures. Anti-seizure medications failed to slow her seizure activity and changing her diet didn't help either, said Peters-Larson. In 2016, neurologists near their Tulsa, Okla., home implanted a vagus nerve stimulator (VNS) but the device did little to reduce the amount of seizures.

After running out of treatment options in Oklahoma, the Peters-Larson family then turned Le Bonheur for help. When Neurologist Stephen Fulton, MD, suggested that Maelee enroll in the hospital's new fenfluramine drug trial, Peters-Larson signed up.

"We were excited about enrolling because nothing else was helping," Peters-Larson said. "We needed to try something else."

After adjusting the dosage of her new medication, Maelee was seizure free for 31 days – her longest stretch ever. Maelee's cognitive abilities also have improved.

"Before she started taking (fenfluramine), she never had a day without a seizure," Peters-Larson said. "Without that constant electrical storm in her brain, she can now learn and retain information."

In addition to taking fenfluramine, patients enrolled in the trial must also continue to take their anti-seizure medications and are required to return to Le Bonheur for monthly study visits.

"There has been much work in the last 10 plus years convincing the FDA that a partial seizure in a 4-year-old has the same underlying mechanism as it does in an adult and adolescent," said Wheless.

The device has been life-changing for 10-year-old Oliver Prats of Baton Rouge, La. Before he received a VNS in November 2015, seizures were ruling his life, said his mom, Lacey.

Diagnosed with epilepsy at 6 months old, Oliver's seizures became more frequent and more severe by his fifth birthday. He was on three medications that yielded little control of his seizures.

"He wasn't learning and was having anger outbursts. We had lost him to seizures," Lacey said.

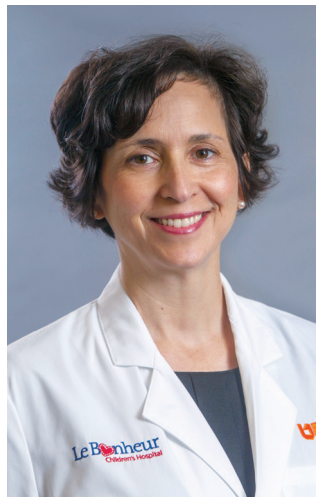
The Prats family was referred to Wheless in 2015. When Wheless suggested a VNS device, the family was hesitant. When new medications and diet changes produced little success, the Prats finally agreed to proceed with the surgery.

Today, Oliver is 15 months seizure-free. He was able to transfer from his special needs school and is thriving, says Lacey.

"He's back to his happy, go-lucky self. It's the best decision we've ever made and has completely changed our lives," said Lacey.

Le Bonheur opens Brachial Plexus Clinic

Le Bonheur's Neuroscience Institute recently launched its new Brachial Plexus Clinic. Led by Pediatric Neurologist Elena Caron, MD, the clinic sees patients who suffer from a brachial plexus injury and who have a limited range of motion in their arms or shoulders, have arm weakness or intense arm or shoulder pain.



Elena Caron, MD

The clinic is designed to help patients manage their condition through a variety of treatment options, including physical therapy, occupational therapy or surgery. Brachial plexus is a broad term that describes a group of nerves that branch from the spinal cord to the neck and travel down the arm. The nerves provide feeling in the arm and also control the muscles in the shoulder, elbow or wrist and hand.

Although brachial plexus injuries typically occur in neonates, an injury can occur at any age. Some common causes of brachial plexus injuries are trauma to a person's shoulder or inflammation, said Caron. Minor brachial plexus injuries can

heal within weeks but more serious injuries may require surgery. If left untreated, a brachial plexus injury can lead to permanent disability in the arm.

"A brachial plexus injury can leave you with a shorter arm, significant pain, decreased function in that limb," Caron said. "The goal of neurosurgery and orthopedic surgery is to restore the best function possible and to eliminate any pain the patient may have."

After diagnosis, a comprehensive treatment plan will be developed, which may include frequent follow-up appointments with physical and occupational therapists or, in more severe cases, surgery, Caron said.

The Brachial Plexus Clinic is held in conjunction with the Cerebral Palsy Clinic and meets twice a month.

Le Bonheur's Brachial Plexus Clinic:

- Led by Neurologist Elena Caron, MD
- See patients with limited range of arm or shoulder motion, arm weakness or instead arm/shoulder pain due to brachial plexus injury
- Held twice a month

DeCuyper joins neurosurgery team

Michael DeCuyper, MD, is Le Bonheur's newest neurosurgeon. He treats both adult and pediatric patients and specializes in minimally invasive neurosurgery.

DeCuyper's research interests include exploring the complex molecular biology of glioblastoma and medulloblastoma.

DeCuyper completed a fellowship in minimally invasive neurosurgical oncology and complex endoscopy in Sydney, Australia, and a fellowship in pediatric neurosurgery at Le Bonheur Children's Hospital/St. Jude Children's Research Hospital.

He is an assistant professor of neurosurgery at the University of Tennessee Health Science Center and a neurosurgeon at Semmes Murphey Clinic.



Michael DeCuyper, MD

IN BRIEF

Neuroscience Institute hosts Facebook Live sessions for families

Le Bonheur's Neuroscience

Institute has recently hosted a series of Facebook Live sessions for families.



The virtual discussions allow families an opportunity to have their questions answered live, on air by a Le Bonheur pediatric expert. Topics have included sleep medicine, other therapies for epilepsy, Batten disease, epilepsy treatment devices and tuberous sclerosis complex. To view previous Facebook Live sessions, visit www.lebonheur.org/NeuroLive.

Patterson joins Neuroscience team

Neurologist Amy Patterson, MD, recently joined Le Bonheur Children's Neuroscience Institute.

Patterson received her medical degree from the University of Louisville School of medicine

and completed a fellowship in child neurology at University of Tennessee Health Science Center. She specializes in epilepsy, tuberous sclerosis complex, neonatal neurology, epilepsy in infancy and general pediatric neurology.



Amy Patterson, MD

Save the Date

12th Annual
Pediatric Neurology Symposium
April 13-14, 2018

The symposium, directed by Dr. James Wheless, will be designed to encompass state-of-the-art practices and trends in treating pediatric neurology patients.

Registration will be available in early 2018. Please watch www.methodistmd.org/cme or call 901-516-8933 for the program.

Le Bonheur
Children's Hospital

Brain Waves is a quarterly publication of the Neuroscience Institute at Le Bonheur Children's Hospital. The institute is a nationally recognized center for evaluation and treatment of nervous system disorders in children and adolescents, ranging from birth defects and learning and behavioral disorders to brain tumors, epilepsy and traumatic injuries.

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Le Bonheur one of first to offer Brineura™, Spinraza™

Le Bonheur is one of only a few children's hospital to offer treatment for rare neurodegenerative diseases, Batten disease and spinal muscular atrophy. Brineura™, an enzyme replacement therapy approved by the U.S. Food and Drug Administration (FDA) in April, is the Gieselmann family's only hope for slowing the progression of 5-year-old Elle's Batten disease — a diagnosis that leads to progressive loss of motor and cognitive functions and seizures.

Batten disease claimed the life of Elle's older sister, Milla, last year. Until the drug's recent FDA approval for neuronal ceroid lipofuscinosis (CLN type 2) Batten disease, Elle participated in its clinical trial at Nationwide Children's Hospital in Columbus, Ohio. The Gieselmanns, who live in Memphis, are relieved Elle can now receive Brineura™— administered twice a month — close to home.

"Being able to stay home will not only help Elle and her recovery from infusions, but it is a game changer for our family as we continue fighting through each day," said Elle's dad, Frazer.

Le Bonheur is committed to helping families who face a rare, devastating diagnosis find hope, says Neuroscience Institute Co-director James Wheless, MD. The hospital is also one of only a handful of children's hospitals to offer Spinraza™, a



Elle Gieselmann, 5, received her first dose of Brineura™ at Le Bonheur Children's on July 20. The drug was approved by the FDA in April for the treatment of CLN type 2 Batten disease.

groundbreaking new drug for spinal muscular atrophy — an often fatal genetic disease that affects muscular strength and the child's ability to move and even breathe.

"Offering innovative therapies for children facing rare diseases is important, particularly when they have few other options," Wheless said. "At Le Bonheur, we have all the resources in place to deliver cutting-edge treatment options to families."