Stem cells could boost this Maryland baby's heart and chance for a normal life

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Surgeons trying a new way to save the life of a baby born with half a heart stood over her open chest and waited for the FedEx box.

Doctors in Miami had overnighted two small vials of stem cells to the University of Maryland Medical Center. Now the Baltimore surgeons would inject the cells, derived from a donor's bone marrow, into the tiny, defective heart of 4-month-old Autumn.

The Hagerstown girl was born with an often fatal condition called hypoplastic left heart syndrome, in which the left half of the heart is unable to pump blood. She is the fifth child to get the injections into the working right half of her heart as part of a national study at the Maryland hospital aimed not only at saving lives, but also allowing children with the condition to live more normally.

"Not to be superstitious, but we liked that she was No. 5," Wayne Brown, Autumn's father, said before the surgery recently.

Both Brown and LeeAnn Janes, Autumn's mother, were born on the fifth day of the month.

"She's become our Lucky No. 5," Brown said.

After seeing some promise in trials on adults who have had heart attacks, strokes and other cardiac problems, researchers believed stem cells might benefit children with heart problems.

Autumn Brown is one of 10 babies in the study now receiving cells to check for safety and feasibility. Another 20 will be enrolled at Maryland and other pediatric hospitals around the country. Half will get stem cells, in a trial to determine whether the treatment helps.

Some 950 babies each year are born with hypoplastic left heart syndrome. Left untreated, the condition is always fatal.

For now, the lone treatment is a course of surgery developed 30 years ago. It's performed in three parts: at birth, at about four months and at age two or three.

Even with the surgeries, only 60 percent of the children celebrate their fifth birthdays. Then the overtaxed partial heart eventually fails, necessitating a heart transplant, which brings more risks.

Now doctors in the study are injecting the stem cells into the working half of the heart during the second surgery.

Autumn is a plump and cheery bundle. The only outward clues that she suffers a disorder are an oxygen line to her nose and a port for a feeding tube.

Dr. Sunjay Kaushal, director of pediatric and adult congenital surgery at the University of Maryland and one of the nation's foremost surgeons for hypoplastic left heart syndrome, said Autumn's general health beyond the condition and her supportive parents made her a good candidate for the stem cell study.

His team of about a dozen arrived early for the surgery in mid-February, draped blue cloths around Autumn and hooked her up to machines that would at times do her breathing and pump her blood for her. Her pacifier and stuffed monkey were carefully stored and labeled in a bag

and set aside. Her chest was reopened along the same several-inch-long line used in her first surgery.

Three doctors aimed their headlamps down at Autumn's strawberry-sized heart.They worked in tandem with the nurses, anesthesiologists and other professionals who packed around the table in the pediatric operating room in the University of Maryland Children's Hospital. Other attendants scurried around the room, keeping track of supplies and other needs of those in the surgery huddle.

The heart surgery done, the team waited for the courier delivering the stem cells.

The roughly 1.67 million cells had been in a freezer at the biotech company Longeveron the day before. They had largely thawed before their scheduled arrival, so too much of a delay could jeopardize their viability.

The opaque liquid cells arrived at the hospital in a toaster-sized cooler pack at about 12:15 p.m. and were hurried into the operating room. They were carefully unwrapped by sterile gloved hands.

A quick examination under the microscope showed they were 77 percent viable, above the 70 percent threshold for use. They were drawn into eight small syringes, which were then laid on a cloth-covered tray next to the surgical team.

A member of the team held up a diagram of a heart for Kaushal, an associate professor of surgery in the University of Maryland School of Medicine and director of pediatric cardiac surgery at the medical center. He pointed to a spot each time he plunged a needle into Autumn's heart.

If the cells work as they did in Kaushal's computer models and lab

animals during nearly a decade of research, they will boost the strength and longevity of Autumn's half a heart.

Kaushal says the first four children to receive the cells—three at Maryland and one at the Johns Hopkins Hospital—were faring well.

"I think this is game-changing for these kids," Kaushal said. "I believe these young hearts are going to be the most responsive."

In a normal heart, the right side of the heart typically has the less stressful job of pumping deoxygenated blood from the body to the lungs. The left side more powerfully pumps the resupplied blood back out to the body.

In babies with Autumn's syndrome, the right side has to do double duty.

The three surgeries change how blood flows so deoxygenated blood goes right to the lungs and refreshed blood is pumped around the body by the right side of the heart.

Kaushal has been working with Dr. Joshua Hare, the former Johns Hopkins researcher who became the founding director of the Interdisciplinary Stem Cell Institute at the University of Miami Miller School of Medicine. Hare formed Longeveron to translate research into medical use. Longeveron, the University of Maryland, and the state-established Maryland Stem Cell Research Fund are sponsoring the initial $1.5 million cost of the study.

Longeveron harvested the mesenchymal stem cells from a young adult donor and allowed them to multiply in a culture to produce enough for use in every baby in the trial. These types of cells can become any kind of human cell.

Once in a baby's heart, Hare said, they can reduce scar tissue, reduce inflammation, promote new small vessels and—perhaps most importantly—stimulate the heart to grow.

Data collected so far on stem cells in adults or children have not convinced everyone of the long-term possibilities. Clinics that have exploited loopholes to use unsanctioned stem cell therapies have drawn negative publicity.

The U.S. Food and Drug Administration has warned consumers about unintended and harmful consequences. The FDA has approved stem cells only for disorders related to blood production.

But the agency has allowed their use in well-reviewed studies, including the heart study at Maryland.

"In my experience, it's a highly promising therapy and people do well, and we just need more data to prove it," Hare said.

"My ultimate hope is we can show we can reduce the need for transplants and kids do better, not just by increasing the functioning of their hearts but make them feel better and live longer," he said. "The goal is really to make kids better."

Autumn's mother wants nothing else for her daughter. The girl was discharged, then returned to the hospital with a complication. Now she's improving and should head soon to a relative's home with her parents and other family.

Janes said she sees the stem cells as "creating a super heart."

And for her part, Lucky No. 5 may have earned more good energy during her surgery: The injections of stem cells began at 12:53 and

ended at 12:58—exactly 5 minutes.