In a parking lot populated by a dozen or so hundreds-of-horsepower cars and SUVs also sit a couple of one-horsepower conveyances: Amish buggies. This is the Clinic for Special Children in Strasburg, Pa. Amish and Mennonite men constructed the clinic here in 1991 and expanded it in 2001.

On the front porch, a docile black dog named Bowie greets patients and visitors. Inside are women wearing unadorned clothes and bonnets. (The Amish, Mennonites, and others who settled the area in the 1700s in search of a humble life and religious freedom often call themselves "the Plain People.") Children play in the waiting room. One child roots through a toy chest full of Beanie Babies. Sunlight streams into the building from 78 windows. Down a hallway where skylights brighten the oak floor and fir beams (held together with wooden pegs rather than nails, the custom for Amish and Mennonite construction), and past a genetics lab, a family sits in an alcove in close conversation with physician Kevin Strauss.

The family is Mennonite. They are from southern Alabama, not far from the Florida panhandle. Their baby daughter is squirming on her thin father's lap. The girl has a rare genetic condition called maple syrup urine disease (MSUD). It is more common by orders of magnitude among Amish and Mennonites—the ratio is one in three-hundred for them and perhaps as high as one in a million for the population at large, says Strauss. MSUD, which gets its name from the sweet smell of the afflicted child’s urine, is a metabolic disease that causes amino acids to accumulate in the body. It can lead to brain swelling, neurological damage, and death. The only cure is liver transplantation. Although MSUD is not a liver problem per se, a new liver compensates for the lack of critical enzymes normally found throughout the body. Almost immediately after transplantation, an MSUD patient’s metabolic health reverts to normal.

The Clinic for Special Children, cofounded by West Virginia native and Harvard-trained physician Holmes Morton and his wife, Caroline Morton, is one of the best places to get diagnosis and treatment for genetic disorders particular to the Amish and Mennonites, including MSUD. Children’s Hospital of Pittsburgh of UPMC has become the clinic’s go-to hospital for the curative transplant.

Holmes Morton, who cofounded the Clinic for Special Children (shown left and right) near Lancaster, Pa., now refers all of his transplant patients to Children’s Hospital of Pittsburgh. In the past three years, Pitt surgeons have performed 38 life-enhancing liver transplants on clinic children.
Of course, transplantation has its risks. In addition to challenges presented by the surgery itself, patients must perpetually take immunosuppressants. Yet Pittsburgh boasts an overall survival rate among pediatric patients of 83 percent 10 years after a liver transplant. And all 38 of the Clinic for Special Children's patients transplanted at Children's—28 because of MSUD—are thriving.

The Alabama family has come to the clinic to find out for themselves about the benefits and pitfalls of transplantation. The young father (the family asked that their names not be used), dressed in black trousers and a white button-down shirt with a cell phone hanging from his belt, says to Strauss, “My opinion was [the Mennonite community] doesn’t do transplants.” That was, he adds, until a member of a Mennonite family close to his did.

The father came prepared with a list of questions regarding mortality on the operating table, immunosuppression, rejection, maintenance of MSUD through diet, and cost. (Mennonites and the Amish typically do not carry health insurance. It’s a pay-as-you-go process for them, with material help given by friends, family, and other members of the community. The cost of a liver transplant, Strauss says, is equivalent to about a decade of typical treatment, and treatment can go on for 30 or 40 years or longer.)

As the father talks, a toy placed next to the band of preschoolers sitting on the floor erupts into a rousing rendition of “London Bridge.” Amid the commotion, Strauss does his best to allay the man’s fears.

“One thing we can tell you—one thing we are obligated to tell you—is that [transplantation] cures MSUD in every case and cures it 100 percent,” Strauss says. “There’s no longer a need for a special diet, there’s no need for blood testing. That’s it.”

What that means, Strauss tells the father, is normalcy. There will be no metabolic crisis, no dietary restrictions (MSUD patients have to carry a special dietary protein powder with them at all times), and no risk of MSUD-related brain damage or death. There can and likely will be medical problems related to the transplant, he says, but “it’s a good trade-off.” To reap these benefits, Strauss continues, leaning forward, “You have to go where they know what they’re doing. That’s Pittsburgh. You have to go to Pittsburgh. We’re partners. They can manage any crisis.”

The clinic’s relationship with Pitt has taken root throughout the past three years under the direction of George Mazariegos (Fel ’93), an MD associate professor of surgery at Pitt and director of Pediatric Transplantation at Children’s. Morton and Strauss worked with him—along with colleagues such as Jerry Vockley, MD/PhD chief of the Division of Medical Genetics at Children’s and professor of pediatrics and human genetics at Pitt, and David Finegold, an MD professor of pediatrics, medicine, and human genetics at Pitt—to develop transplant protocols to deal with and stave off the metabolic crises potentially faced by MSUD sufferers.

Strauss acknowledges there is resistance to transplantation in certain segments of the Mennonite and Amish communities, particularly those near the clinic.

“From the beginning, there’s been opposition. People have felt that Dr. Morton and the clinic are right here and can manage MSUD. But Dr. Morton and I both know we can’t fully protect anyone from the risks of MSUD,” Strauss tells the family.

The father turns to his wife, then looks back to Strauss and says, “We’re going to go home and think.”
Dustin Hahn and his parents, Dawn and Kevin Hahn, have already made their decision. Dustin will have his transplant. A cheery homeschooled seventh-grader from Lititz, Pa., who’s quite comfortable in the presence of adults (perhaps from a lifetime of dealing with doctors), Dustin came to the conclusion he was ready as the 2006–2007 ice hockey season approached.

“He asked Dr. Strauss if he could play on a hockey team,” Dawn Hahn says. She reports that Strauss said Dustin could, but it would mean a lot more work to keep him healthy, because soft bones are a consequence of MSUD.

As the Hahns discuss Dustin’s potential hockey career (he’s a Philadelphia Flyers fan), Strauss joins them in the waiting room. Picking up on the conversation, Strauss says, “Hockey for [Dustin] is just the tip of the iceberg. There are benefits in terms of attention and education and employment. Adults with MSUD don’t take as good care of themselves as their parents did when they were children. There are mood disorders to consider. About 30 percent of adults with MSUD are on antidepressants—that’s three or four times higher than the general population.”

Dustin has been on the liver transplant waiting list since January. Since then, Children’s has called twice telling the Hahns a liver was available, but both times they got a follow-up call before they could make the four-hour drive to Pittsburgh. The organs were not suitable for transplant, they were told.

“I was excited,” Dustin says, “and a little scared. My heart went down when we found out it wasn’t going to work, but I got over it. It’s practice.”

“We don’t want a lot more practice,” his mother chimes in.

So Dustin waits for his opportunity to eat “regular” food (he wants a bacon cheeseburger and a Frosty from Wendy’s), play sports, and be able to concentrate better in school. He is hopeful, and, in fact, his greatest fear seems to be that the doctors and nurses at Children’s will try to turn him into a Pittsburgh Penguins fan. “Flyers all the way,” he says.

Dustin Hahn received his transplant shortly before Pitt Med went to press.