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INNOVATIONS: NICHE DRUGS

Families fund a search for rare disease's cure

Group partners with biotech firm to fight ailment drug giants avoid

By Jon Van
Tribune staff reporter
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Large drug companies relish male impotence, high blood pressure and arthritis because the millions of people with those afflictions are a lucrative market.

But with a genetic ailment that strikes only one child in 6,000, the prospects for profit are slim. And even if a pharmaceutical company hits pay dirt after spending millions in research and development, there aren't enough customers for the treatment to justify the expense.

That's why research under way in the Woodridge-based laboratory operated by Decode Genetics Inc. is so different--and personal.

Seeking treatment for a rare, often fatal, genetic disorder that attacks children has motivated researchers because Decode's clients are the patients' families. The scientists and Libertyville-based Families of Spinal Muscular Atrophy have discovered drugs that may alleviate SMA, which inhibits control of muscles in the limbs, neck and chest.

The partnership is "something you wouldn't see in many places outside the United States," said Dr. Kari Stefansson, chief executive of Decode. "Relatives of people with a rare disease aren't going to sit by. It's difficult to get government or large companies interested."

Stefansson, who treated SMA patients when he was on the University of Chicago faculty in the 1980s, said the families' devotion inspires Decode's scientists.

Decode is an unusual biotech firm. It is based in Reykjavik, Iceland, in order to combine centuries of family data about the Icelandic population with modern genetic research to discover new therapies.

"Decode is a hybrid of American and Icelandic cultures," said Stefansson. "Our roots in our community are deep."

Decode's Woodridge chemical labs do research to support the parent company's drug exploration. It also handles contract research for drug companies and biotech firms.

But the research for Families of SMA is different.

The SMA group, which started 20 years ago and consists of 5,000 families, has raised more than \$12 million from endeavors that range from selling lemonade to hosting golf tournaments. It has a three-year, \$5.2 million agreement with Decode to discover and produce SMA therapies.



"We totally had no choice," said Audrey Lewis, executive director of the group and the mother of a son with the affliction. "Families are losing children and losing their struggle with the disease. Children are getting weaker."

From the first, the organization allied itself with academic researchers studying SMA, Lewis said, and formed a scientific advisory board. With the money the organization raises it seeks research proposals, much as the National Institutes of Health would.

Earlier work with academic scientists and a California biotech firm led to identification of the genetic defect that leads to SMA. That laid the groundwork for Decode chemists to search for a substance that will correct the defect.

It's a terribly difficult quest.

"This isn't a typical drug discovery effort," said David Zembower, Decode's vice president for chemistry. "You have a genetic mutation that results in producing a protein that's deficient. The protein is involved with motor neuron pathways, but its precise action isn't known.

"Usually in drug searches, you're looking for some way to knock down a protein's action, but here the challenge is to find a small molecule that will fix the protein or help the cells increase the number of good proteins they produce."

Research has identified some drugs already on the market that may help do this, and Families of SMA is working with medical centers to test them in humans. Meanwhile, Decode chemists have engineered chemicals they expect would have a greater effect in producing desirable target proteins, and those substances are being tested in animals.

While this progress provides hope to the families, the risks are so high that most pharmaceutical firms would decline to fund such a long shot. For one thing, the research assumes that producing a higher amount of the protein, called survival motor neuron protein, will reduce or eliminate symptoms for the patients.

The assumption has not been proven.

Animal tests now under way should indicate if the hypothesis behind Families of SMA's drug development effort is sound, said Chris Spancake, the group's research director. If it is, and the researchers can get approval from the Food and Drug Administration, the group will have clinicians ready to begin tests in human patients.

"We're not interested in making money. We just want to find a treatment," said Spancake, who lost a child to SMA.

Unlike the usual dry status reports delivered at scientific meetings, the relationship between Families of SMA and its researchers is far more personal. Family representatives regularly attend meetings where researchers report their findings.

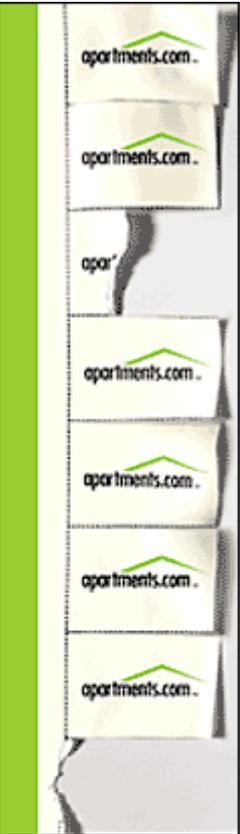
"Scientists meet the kids in the wheelchairs motoring around the halls, and we get an opportunity to talk with the older children," said Mark Gurney, Decode's drug discovery vice president. "We have chemists working in Chicago and biologists working in Iceland, collaborating on this project.

"When some families with SMA children in Iceland heard about the project, they raised money to send to Chicago. We invited them to our headquarters and about 30 people came with their kids.

"They raised money by selling T-shirts, and many of our employees got them. It's been a source of inspiration to everyone."

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