

# Clinical trials: Putting you on the forefront of medicine

*Local companies constantly looking for volunteers to help in the discovery of new drugs*

By Amy Cavalier

Take a look in your medicine cabinet or around your local pharmacy. All those pills, creams and other medications you see have gone through years of testing and research before being approved by the U.S. Food and Drug Administration and making it onto the shelves.

That's where you come in.

Without volunteers to participate in clinical trials, there would be no new medicines, said Adam Larrabee, director of business development for Rochester Clinical Research.

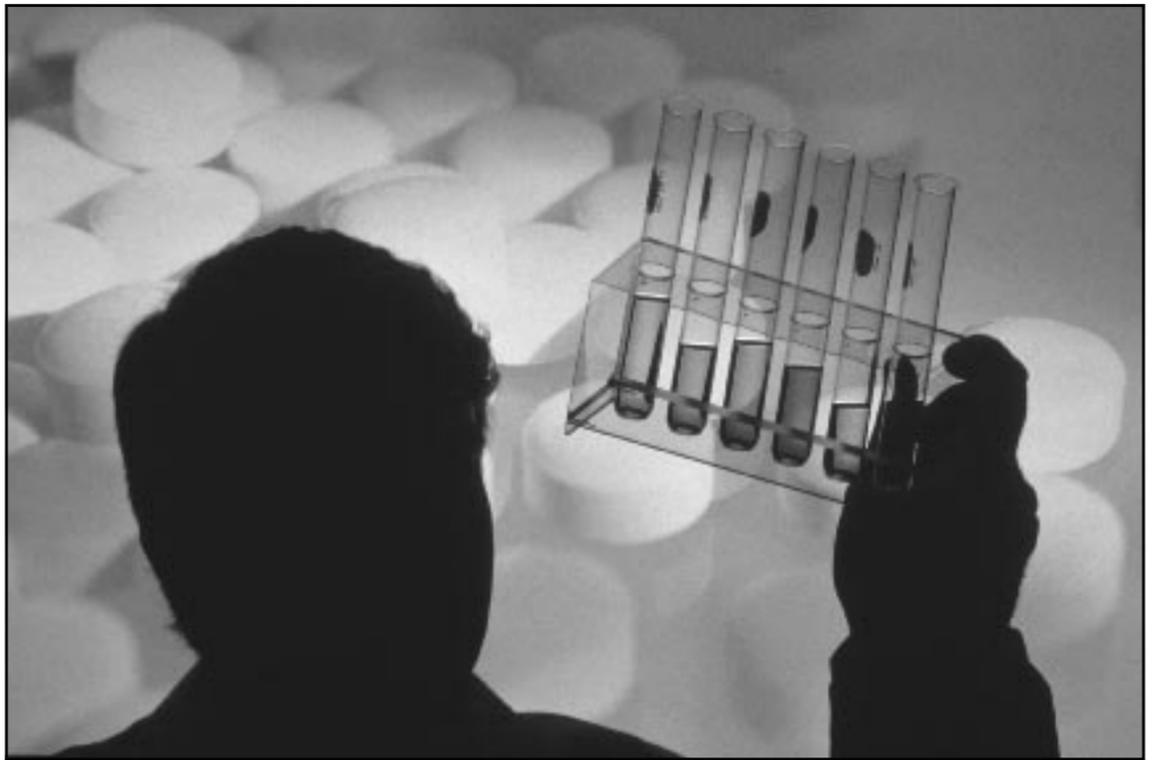
"I think it's interesting that in a poll done in 2008, only 50 percent of Americans say that health-related research has helped them or someone close to them, but 90 percent of those people purchased prescription drugs for themselves or someone in their family," said Larrabee, citing a study by Research! America.

You've probably seen an advertisement for a clinical trial before in local magazines or newspapers, including this "In Good Health," offering you

and Drug Administration.

Businesses like Rochester Clinical Research focused solely on conducting clinical trials. Rochester Clinical Research conducts about 40 trials each year. Other medical facilities conduct clinical trials in addition to providing care to regular patients, such as AAIR and Skin Search of Rochester, Inc. A partner of Dermatology Associates of Rochester, Skin Search conducts anywhere from 15 to 20 trials a year, while AAIR conducts about 25.

Besides bringing in revenue, participating in clinical research give doctors the opportunity to stay on the cutting edge of medical research, said Becky Ricotta, research administrator for AAIR. It also gives them the chance to offer something to their patients beyond what is currently available.



ers people do it for the money, which is intended to reimburse the patient for travel and time. Some trials can go on for just a few months, for example a study on a drug to relieve ragweed allergies, while others can extend over years, such as one on lupus.

Another benefit to being part of a clinical study is that the patient gets a lot of one-on-one time with doctors.

meet very specific criteria.

"For every protocol the sponsor is looking for a very specific type of patient," said Ricotta. "They need to show that their drug is going to work, so everybody has to have some kind of symptoms. They usually have to be on a certain type of medication, so we go through a lot of people before we find the right fit for a study."

The factors that allow someone to participate in a clinical trial are called "inclusion criteria" and those that disallow someone from participating are called "exclusion criteria," according to ClinicalTrials.gov. Criteria include things such as age, gender, the type and stage of a disease, previous treatment history, and other medical conditions. Some studies are looking for participants with specific illnesses or conditions, while others need healthy participants.

Larrabee said the more specific the criteria, the more difficult it is to find people.

"We have a data base of over 18,000 people who have asked about being involved in a study at one point or another," he said.

There are four phases in clinical trials.

In phase one, 20 to 100 healthy volunteers are given incrementally larger amounts of the study drug to test its safety, tolerability and to determine other aspects of how the drug reacts with the body, according to Conducting Clinical Research by Judy Stone, a medical doctor. In phase two, the drug company, or sponsor, determines the efficacy for the drug's intended use and tries to find the best dosage. Phase three broadens the population that receives the new drug, including some real-world patients who do have other medical problems. In phase four,



Ricotta



Larrabee



Forster



Loss

money to participate in a study for anything from diabetes to excessive sweating, acne and arthritis. According to three Rochester-area medical research centers, there are many questions and misconceptions about participating in a clinical trial. There are also many benefits.

"A clinical trial is a controlled scientific study done with an investigational medication to test the medication's effectiveness and safety on human beings," according to Allergy, Asthma, Immunology of Rochester, P.C.'s website, also known as AAIR. According to AAIR's website, medications are tested in the laboratory and through animal research for years before trials are conducted on humans. Clinical trials are required by the Food

"There is a benefit to the community to give patients the opportunity to try out new treatments for their disease that they otherwise would not have the opportunity to do so," said Janice C. Loss, a registered nurse and director of operations for Skin Search.

Ricotta said there are several reasons people chose to be part of clinical research trials. Some people do it to ensure there will be medications on the market to help future generations.

"A lot of people who have asthma have children or grandchildren with asthma, so they want to help," she said.

Other people do it because they aren't finding relief for their diagnosis through drugs currently on the market. Some volunteer because their primary care physician recommends it, and oth-

"People like to be in clinical trials, a lot of the time, because they feel like they're getting taken care of," said Ricotta. "Sometimes you wait to see your doctor for an hour and then you're in there for five minutes. It's not like that in a clinical trial. You never have to wait and then be rushed out of here."

Ricotta said trial participants are encouraged to talk to their primary care physician about being in the study.

"We also share the information with their primary care physician; lab results, blood work, EKGs. We send them copies so they don't have to repeat that," she said.

Not everyone is qualified to participate in a clinical study. Volunteers must

further safety and efficacy data is gathered, and the studies may be expanded to target new groups of ages, sexes or ethnicities.

After the drug is marketed, if surveillance for safety uncovers serious side effects that have not been previously recognized, it could result in the drug being removed from the market or warnings being added. Since 1975, 22 drugs have been removed from the market after FDA approval, according to a 2002 report by the FDA and Center for Drug Evaluation and Research.

"There's no guarantee when you do a clinical study that you're going to get medicine and get better, but there's overall benefits" said Denise Forster, assistant director of Skin Search. "We find out about new medicines and can assess the safety of those medicines."

Clinical trials are monitored throughout the entire process by the FDA and the Institutional Review Board.

"Their job is to protect the rights of the subject in the study, so they have to approve everything and watch over the study to make sure patients' rights are not violated," said Ricotta.

Confidentiality is paramount in clinical trials, said Forster. Patients in a clinical trial are assigned a number.

Sometimes, a clinical trial may reveal that a drug intended for one purpose is also helpful for treating another

## Local companies conducting clinical trials in Rochester

For more information on clinical trials in your area, visit:

[www.aair.info](http://www.aair.info) – Allergy, Asthma, Immunology of Rochester, P.C.

[www.skinsearch.org](http://www.skinsearch.org) – Skin Search of Rochester, Inc.

[www.rcrclinical.com](http://www.rcrclinical.com) – Rochester Clinical Research

[www.clinicaltrials.gov](http://www.clinicaltrials.gov) – a service of the U.S. National Institutes of Health

condition. Larrabee said that drug would have to be studied again before it could be approved for the alternative use. In some cases, a sponsor may end a study before it is complete, either due to patient safety concerns, or because the drug isn't performing as hoped.

"Getting a drug to market costs billions of dollars, so if it's not going to make it, a lot of times they'll just cut their losses," Ricotta said of the research sponsor.

Larrabee said it can cost up to \$25,000 just to get one research site started. A phase three study, which is the last phase before approval, can require upwards of 300 sites.

And not all drugs tested make it to market.

"There's a high failure rate," said Larrabee, "that's why there's so many studies."

# Coping with Celiac Disease

Many of those who suffer from the disease don't know they have it

By Deborah Jeanne Sergeant

For two years, Cassandra Coffey had tried high potency iron supplements to combat anemia but with no success. When a dietitian friend heard of her other complaints — including irritable bowel, weight loss, joint/muscle pain, and aphthous ulcers (also known as canker sores) — she gave Coffey information on celiac disease.

Someone with celiac disease is unable to digest wheat gluten, which results in a plethora of uncomfortable side effects such as intestinal problems and, if left untreated, some very serious problems, including rickets, osteoporosis, infertility, and lymphoma of the intestine.

Only 1 percent of the population has celiac disease, but only 10 percent of those are diagnosed. It doesn't always present with gastro-intestinal problems, which can make diagnosis tricky.

For Kathy Oaks, the Rochester Celiac Support Group's vice president, celiac disease went undiagnosed for decades.

"My father has celiac disease and I never considered it as a cause of my arthritis, thyroid issues or hives because my father had the severe gastrointestinal issues and I had none," she said. "I also was not underweight."

When her family doctor suggested a blood test, Oaks agreed and was later surprised to learn that she tested positive.

After reading up on it, Coffey suspected she could have celiac. The blood work and a follow-up endoscopy confirmed the diagnosis. Typical to celiacs, she received a bone scan after diagnosis that revealed osteopenia, the precursor to osteoporosis.

Subsequent testing of her first-degree relatives showed that Coffey is the only one in her family with celiac. Her children, ages 8 and 10, along with her husband, siblings and parents, tested negatively.

With no known cure for celiac disease, the only treatment is avoiding gluten, a protein found in wheat, rye and barley. By adhering to a gluten-free diet, Coffey found after six months that her iron levels returned to the normal stage with only a normal multi-vitamin (not the high-level supplement she had been taking).

Despite regular exercise, Coffey had always felt exhausted. After going gluten-free for six months, her energy level improved.

"I remember running up the steps with a laundry basket and thinking when I got to the top, 'I'm not out of breath!'" she said.

Some of Oaks' rheumatoid arthritis symptoms diminished noticeably after going gluten-free five years ago; however, "if I get the smallest amount of gluten, I have severe abdominal pain and diarrhea within two hours," she said. "It is usually from cross contamination because I am very careful about food."

She still suffers from allergies she says were caused by the damage celiac disease caused to her body.

Four years after her diagnosis, as president of the Rochester Celiac Support Group ([www.rochesterceliacs.org](http://www.rochesterceliacs.org)), Coffey guides others through the

adjustment of living gluten-free by sharing what she has learned.

"I stay informed about questionable ingredients that might contain gluten so that I am able to read food labels and determine whether or not a food is safe," she said. "I eat at restaurants only rarely. I don't think this is entirely necessary to be gluten-free, but it's what is comfortable for me. I have the celiac panel blood test periodically to make sure my antibodies are within normal limits."

It's not simply a matter of avoiding bread, pasta, crackers and baked goods. Many processed foods contain flour such as some brands of ice cream, marshmallows, salad dressing and candy. Most restaurants thicken gravy and sauces with flour instead of the more expensive corn starch (which is naturally gluten-free).

"The diet can be overwhelming at first, trying to learn everything all at once," Coffey said.

She tells newly diagnosed celiacs, "It will get easier and feel like less work as you get used to it. Take the time to find substitutes for the gluten-containing foods that you love. When you know we can have these replacement foods, you never feel like you are deprived."

Of course fruits, vegetables, meat, and dairy items naturally do not contain gluten; however, if these items are



Kathy Oaks and Cassandra Coffey

for them and a gluten-free pizza for myself.

I do keep regular wheat bread for my family's lunches, they have wheat-containing cereals for breakfast, and we have other gluten-containing foods in the house. But, I don't keep any wheat flour because it's too dusty. If I bake, it's gluten-free all the time."

Twenty-five years ago, gluten-free flours, mixes and prepared foods were scarce and marginal in flavor and texture; however, "it's come a long way as far as the availability of food and the taste," said Judy Mazerbo, owner and manager of Health and Harmony Natural Foods in Victor.

She added that blended flours are available now. Gluten, the problematic protein, makes baked goods palatable in texture. Single-grain flours cannot match up, so gluten-free blends include a few kinds of ground beans, potato starch, tapioca flour, sorghum flour, and fava. Rice flour, although somewhat gritty, can also replace wheat flour in some recipes.

"It depends upon what you're trying to make," Mazerbo said.

Coffey minimizes cross-contact in the kitchen by using one countertop for gluten-containing foods only. All of the other countertops are gluten-free.

She prepares the family's lunches on the "wheat countertop" and organizes her pantry based upon what contains gluten and what doesn't.

Still, Coffey misses egg rolls and beer-battered fish fry. Oaks misses soft ice cream cones, although her favorite ice cream venue keeps a box of gluten-free cones on hand for her.

"Always look on the positive side," Coffey said. "For example, If you are gluten-free and are planning to attend a banquet, don't focus on the foods you won't be able to eat. Instead, focus on the social event and why you are at the banquet in the first place. Is it your child's sports team or a celebration of some sort? That's why you are really there, not for the food. And, do contact the staff in advance so that you will know that there is something for you to eat, even if it's different from what everyone else is eating."

Complementary medicine practitioners of Eastern medicine may offer celiacs other options to a lifelong gluten-free diet. Sonan Targee, an herbalist with Ancient Universal Medicine in Rochester, views celiac disease as "not a single disease," he said. "It causes an impaired absorption of nutrients. The basic treatment strategy is to stimulate the [digestion]."

But his approach is not one-size-fits-all.

"There are so many factors," he said. "It can be hard to put one's finger on and so many variables in a person's life."

The treatment plans depend upon how celiac manifests with a patient and can include taking herbs, engaging in exercise, chewing thoroughly, and reducing stress.

breaded, creamed or otherwise processed with flour, they will.

Oaks cooks mostly gluten-free but still makes bread and pasta for her husband. To make cooking easier, Coffey cooks gluten-free for her entire family, with a few exceptions.

"There are a few gluten-containing meals that I prepare for my family that have gluten, such as pizza," she said. "In this case, I'll cook a regular pizza