To:	PHILIP R. ZENDER(ip-squiretm@squirepb.com)
Subject:	U.S. Trademark Application Serial No. 86854469 - GENE PILL - 120753.00107
Sent:	January 11, 2024 02:06:28 PM EST
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United States Patent and Trademark Office (USPTO)

Office Action (Official Letter) About Applicant's Trademark Application

U.S. Application Serial No. 86854469

Mark: GENE PILL

Correspondence Address:
PHILIP R. ZENDER
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555 CALIFORNIA STREET, SUITE 550
SAN FRANCISCO CA 94104
UNITED STATES

Applicant: Engene Inc.

Reference/Docket No. 120753.00107

Correspondence Email Address: ip-squiretm@squirepb.com

REQUEST FOR RECONSIDERATION AFTER FINAL ACTION DENIED

Issue date: January 11, 2024

Applicant's request for reconsideration is denied. *See* 37 C.F.R. §2.63(b)(3). The trademark examining attorney has carefully reviewed applicant's request and determined the request did not: (1) raise a new issue, (2) resolve all the outstanding issue(s), (3) provide any new or compelling evidence with regard to the outstanding issue(s), or (4) present analysis and arguments that were persuasive or shed new light on the outstanding issue(s). TMEP §§715.03(a)(ii)(B), 715.04(a).

Accordingly, the following requirement(s) and/or refusal(s) made final in the Office action dated May 2, 2023 are **maintained and continued**: Section 2(e)(1) Refusal - Class 42.
See TMEP §§715.03(a)(ii)(B), 715.04(a).

In addition, the following requirement(s) and/or refusal(s) made final in that Office action are **withdrawn**: Section 2(e)(1) Refusal - Class 45.
See TMEP §§715.03(a)(ii)(B), 715.04(a).

Section 2(e)(1) Refusal

The Section 2(e)(1) Refusal is continued for Class 42 only.

As indicated previously, applicant's services include pharmaceutical research and product development services. This encompasses pharmaceutical research and development of pills that feature genes or genetic materials for gene therapy purposes. The Fiercebiotech article notes that applicant plans to use its gene therapy technology "to develop a gene pill that can offer an oral approach to developing a long-acting insulin." The Genengnews article notes that applicant has been developing "gene pill delivery systems" that "could be used to treat a range of immune disorders." Thus, applicant's pharmaceutical research and development appears to be actively focused on gene pills. The evidence supports that the term is also widely used in applicant's industry. The Medscape article cites a Wall Street Journal report informing of "a new technology now under development, aiming to deliver injectable protein drugs using an oral "gene pill" approach." The article indicates, "Inspired by gene therapy, it is called a "gene pill" and contains the gene for a disease-treating protein rather than the protein itself." The Science Daily report informs of approaches to "make gene therapy treatments that are stable enough to take as a pill." The UCSF article informs that "the oral delivery of normal genes has been a long-sought and elusive technique," and is "sometimes referred to as a "gene pill"." The Science.org article titled "A Gene Pill for Lactose Intolerance?" notes of new techniques allowing for people to "one day be able to pop a "gene pill" that offers long-lasting treatment" for particular ailments. In the Euronews article, the gene therapy company MeiraGTx CEO indicates that they "can now put the genes…that control metabolism into the body and give a pill when we want those drugs." The VCU Health articles informs of gene therapies with a goal of developing a pill for the treatment. Based on the evidence from the NIH, gene therapy refers to "a technique that uses a gene(s) to treat, prevent or cure a disease or medical disorder." Applicant's pharmaceutical research and development services could feature pills that use genes to treat disorders, in other words, a gene pill. Thus, the wording immediately conveys information about the services.

Applicant's argument that "pill" has other meanings in this context is not persuasive. Determining the

descriptiveness of a mark is done in relation to an applicant's goods and/or services, the context in which the mark is being used, and the possible significance the mark would have to the average purchaser because of the manner of its use or intended use. *See In re The Chamber of Commerce of the U.S.*, 675 F.3d 1297, 1300, 102 USPQ2d 1217, 1219 (Fed. Cir. 2012) (citing *In re Bayer Aktiengesellschaft*, 488 F.3d 960, 963-64, 82 USPQ2d 1828, 1831 (Fed. Cir. 2007)); TMEP §1209.01(b). Descriptiveness of a mark is not considered in the abstract. *In re Bayer Aktiengesellschaft*, 488 F.3d at 963-64, 82 USPQ2d at 1831. Additionally, as noted previously, applicant has indicated that it "anticipates that its research and development services will encompass pills or pill shaped forms as a delivery method for its therapeutics," its "product development services will encompass pills or pill shaped forms as a delivery method for its therapeutics," and "its product development services, which may be licensed, will encompass pills or pill shaped forms as a delivery method for its therapeutics." Thus, applicant's own statements support the descriptiveness of "pill" in the context of applicant's services.

Partial Abandonment

If applicant does not respond to this Office action by the response deadline, International Class(es) 42 will be deleted from the application. The application will then proceed with International Class(es) 45 only. *See* 37 C.F.R. §2.65(a)-(a)(1); TMEP §718.02(a).

If applicant has already filed an appeal with the Trademark Trial and Appeal Board, the Board will be notified to resume the appeal. *See* TMEP §715.04(a).

If applicant has not filed an appeal and time remains in the response period for the final Office action, applicant has the remainder of that time to (1) file another request for reconsideration that complies with and/or overcomes any outstanding final requirement(s) and/or refusal(s), and/or (2) file a notice of appeal to the Board. TMEP §715.03(a)(ii)(B).

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'Gene Pill' Offers Alternative to Shots

By Sharon BegleyStaff Reporter of THE WALL STREET JOURNAL
June 21, 2005 12:01 am ET

For people who have to inject themselves regularly with insulin to treat diabetes, erythropoietin to treat anemia or other protein drugs for various diseases, there University scientists and a biotechnology company are developing an alternative to injecting the drugs. Inspired by gene therapy, it is called a "gene pill" and contains the gene for a disease-treating protein rather than the protein itself.

Many helpful drugs are actually proteins. But proteins make poor pills, because they are broken down in the gut or poorly absorbed, with the result that they don't deliver the intended benefit. The only choice is to inject them.

However, the body itself makes proteins all the time in cells, following the instructions of genes in the cells. Now researchers are working on delivering genes for medicinal proteins to the body through a pill. A study in lab animals showed that, not only do the genes survive their digestive trip intact, they also get incorporated into cells of the gut -- which then produce the helpful proteins for the body to use.

Although the research is preliminary, with studies in humans still on the drawing boards, outside experts agree it shows promise. The gene pill "could provide an effective alternative method for delivering protein drugs currently administered only through injection," said David Klonoff, clinical professor at the University of California, San Francisco, and editor in chief of the journal Diabetes Technology & Therapeutics, which published the study in its June issue.

Today's protein drugs, such as growth hormone to treat dwarfism and blood issue.

Today's protein drugs, such as growth hormone to treat dwarfism and blood factors to treat hemophilia, have several drawbacks. Patients often skip doses because the drugs have to be injected, rather than swallowed. Also, these proteins are either extracted from human cadavers or animal tissue, which is slow and inefficient, or -- more common -- produced through recombinant DNA, which is expensive. Moreover, injectable drugs are difficult and expensive to store, limiting their use in developing countries.

The gene pill is designed to avoid these problems. The cells lining the intestine are the only ones that take up the DNA for the therapeutic protein, which the cells release into the bloodstream.

The gene itself stays out of the bloodstream, with the result that it can't reach tissues where it might pose a risk. In some trials of traditional gene therapy, in which a virus ferries a therapeutic gene into a patient's cells, the virus has caused dangerous inflammation or disrupted cancer-suppressing genes, causing two deaths and leading the Food and Drug Administration to suspend some

gene-therapy trials in the U.S.

Because cells of the intestine are sloughed off, excreted and replaced every few days, there is little danger that the inserted gene will go astray or deliver too high a dose of the therapeutic protein, says Stephen Rothman, professor emeritus at UCSF and a developer of the gene pill. The pill would be taken every two days or so.

In 1997, Dr. Rothman and three UC colleagues founded Genteric Inc., of Alameda, Calif., which is developing the gene pill commercially. UCSF holds four patents on the gene pill, for which it has granted an exclusive license to Genteric. Dr. Rothman has a financial stake in the closely held company.

In the new study, he and his colleagues gave lab rats and mice several different genes, through a tube. They found that the intestine cells do take up the gene and make the protein, and that they secrete the protein into the blood. When they used the gene for insulin, they showed that the insulin not only gets into the blood but also produces a therapeutic response, in this case lowering levels of blood sugar in rats with diabetes.

"Our approach seeks to avoid many of the problems with current approaches to gene therapy," Dr. Rothman says. In current approaches, once the gene is given to a patient it can't be undone even if it causes harm, as in the patients who developed cancer. In contrast, the effects of the gene pill last only a day or two, until the patient takes another pill.

Experts in gene therapy say they welcome variations on the standard approach. "For relatively small molecules like insulin, this should perhaps work," says Katherine High, a gene-therapy pioneer and professor of pediatrics at the Children's Hospital of Philadelphia.

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Write to Sharon Begley at sharon.begley@wsj.com

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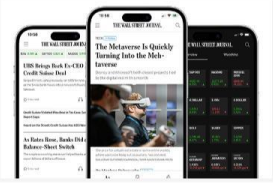

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"Gene pill" could eventually replace injections, WSJ reports

Susan Jeffrey
June 21, 2005

June 21, 2005

New York, NY - A report in today's *Wall Street Journal* reports on a new technology now under development, aiming to deliver injectable protein drugs using an oral "gene pill" approach [1]. *Journal* staff reporter **Sharon Begley** writes, "For people who have to inject themselves regularly with insulin to treat

Diabetes Technology and Therapeutics [2], she writes, shows that "not only

The combination of alcohol and FYCOMPA

Diabetes Technology and Therapeutics [2], she writes, shows that "not only do the genes survive their digestive trip intact, they also get incorporated into cells of the gut—which then produce the helpful proteins for the body to use."

The only cells to take up the gene are those lining the gut, and the protein is released from these cells into the bloodstream, she writes. "The gene itself stays out of the bloodstream, with the result that it can't reach tissues where it might pose a risk," Begley points out. "In some trials of traditional gene therapy, in which a virus ferries a therapeutic gene into a patient's cells, the virus has caused dangerous inflammation or disrupted cancer-suppressing genes, causing two deaths and leading the Food and Drug Administration to suspend some gene-therapy trials in the US."

significantly worsened mood and increased anger. Patients taking FYCOMPA should avoid the use of alcohol. Patients, their caregivers, and families should be informed that FYCOMPA may increase the risk of psychiatric events. Patients should be monitored during treatment and for at least one month after the last dose of FYCOMPA, and especially when taking higher doses and during the initial few weeks of drug therapy (titration period) or at other times of dose increases. Similar serious psychiatric and behavioral events were observed in the primary generalized tonic-clonic (PGTC) seizure clinical trial.

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proceeding with the commercial development of the approach, she writes. "UCSF holds four patents on the gene pill, for which it has granted an exclusive license to Genteric," she writes. "Dr Rothman has a financial stake in the closely held company."

A safety advantage

In their published research report, Rothman et al write that in the new approach, the drug given orally is a DNA plasmid that encodes for the protein of interest. They show in animal experiments that, for example, giving the gene encoding for insulin caused insulin to be secreted, and the available insulin reduced blood glucose levels among diabetic animals.

advantage relative to other gene therapies. "The dose of the drug can be altered from day to day, and treatment can be stopped quickly if untoward side effects are observed," the authors write.

Sources

1. Begley S. "Gene pill" offers alternative to shots. *Wall Street Journal*, June 21, 2005. Available at: www.wsj.com.
2. Rothman S, Tseng H, Goldfine I. Oral gene therapy: A novel method for the manufacture and delivery of protein drugs. *Diabetes Technology and Therapeutics* 2005; 7:549-557. Available at: https://www.liebertpub.com/publication.aspx?pub_id=11. Abstract

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"Gene pill" could eventually replace injections, WSJ reports

Susan Jeffrey
June 21, 2005

June 21, 2005

diseases, there may be hope for an end one day to being a human pin

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Pill-based gene therapy

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A breakthrough in the delivery of gene therapy was recently announced by Genteric, Inc. (http://www.genteric.com). Called the 'Gene Pill', it is a technique for oral delivery of non-viral DNA. The idea behind the pill is that the gastrointestinal organs will convert the introduced DNA to therapeutic proteins that will be distributed naturally by the body. The company tested the method using DNA encoding the human insulin gene. Studies showed that when the engineered DNA is introduced into the body, it is resistant to degradation. When tested in diabetic rats, insulin protein was produced and secreted into the blood stream, lowering the rats' blood glucose levels to normal. The technology has the potential to treat a wide array of other diseases such as growth hormone deficiency. *SG*

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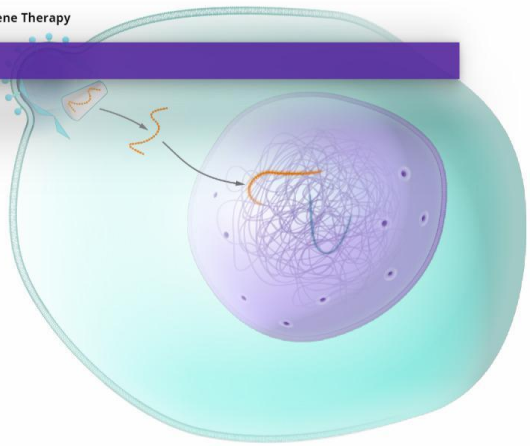


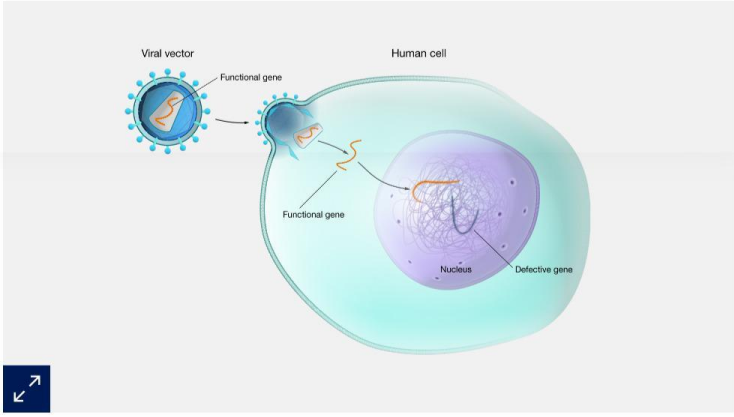
updated: January 9, 2024

Definition

00:00 00:41

Gene therapy is a technique that uses a gene(s) to treat, prevent or cure a disease or medical

disorder. Often, gene therapy works by adding new copies of a gene that is broken, or by replacing a defective or missing gene in a patient's cells with a healthy version of that gene. Both inherited genetic diseases (e.g., hemophilia and sickle cell disease) and acquired disorders (e.g., leukemia) have been treated with gene therapy.



Narration

00:00 00:32

Gene therapy. Gene therapy is a direct way to treat genetic conditions as well as other

conditions. There are also other related approaches like gene editing. There are many different versions and approaches to gene therapy and gene editing. It all rests on understanding how genes work and how changes in genes can affect our health. Researchers all over the world are studying many different facets of gene therapy and gene editing.

Benjamin Solomon, M.D.
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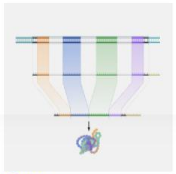

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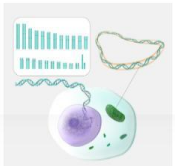

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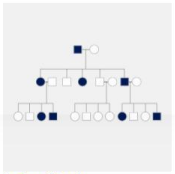

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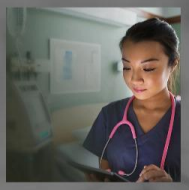


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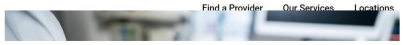
The Forefront of Genomics

FDA-approved treatments use gene therapy to help patients with sickle cell disease

A VCU School of Pharmacy expert breaks down how gene therapies work to relieve pain for sickle cell patients and discusses other potential treatments on the horizon.

December 18, 2023



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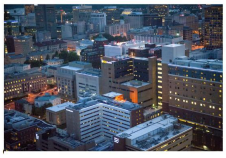

Reflecting on VCU Health's unstoppable commitment to innovative patient care

December 19, 2023

Sickle cell disease affects about 100,000 people in the United States and millions more across the world. The genetic disorder causes red blood cells to form into rigid "sickle" shapes, resulting in severe pain and organ damage, among other health issues. (Getty Images)

By Olivia Trani

The U.S. Food and Drug Administration recently approved two gene therapies, Casgevy and Lyfgenia, to treat sickle cell disease in people ages 12 and older. One of the therapies, Casgevy, use a novel gene-editing technique called CRISPR/Cas9, which can modify a cell's DNA at a targeted location to turn certain genes on or off. The decision marks the first time that a gene-editing treatment has been approved for any human illness.

Sickle cell disease is an inherited blood disorder affecting about 100,000 people in the United States. Individuals with this genetic illness experience debilitating levels of pain and irreversible damage to their organs. The disease disproportionately affects African Americans, and medical research on treatments historically was neglected and underfunded due to biases toward race and ethnicity.

"Despite the fact that sickle cell disease was the first to be understood at the molecular level, patients had limited treatment options for several decades," says **Martin Safo**, Ph.D., a professor in the Department of Medicinal Chemistry at **Virginia Commonwealth University's School of Pharmacy** who has been working on drug discovery for sickle cell disease for almost 30 years.

The FDA approved the two gene therapies after clinical trials demonstrated that 93.5% and 88% of participants reported no pain for one year following treatment. One of those participants is **VCU Health patient named Walter Davis**. He received care from **India Sisler**, M.D., and **Elizabeth Krieger**, M.D, at

Promising new sickle cell therapy trialed at Children's Hospital of Richmond at VCU and VCU Medical Center

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Children's Hospital of Richmond at VCU's **pediatric comprehensive sickle cell center**, as well as **Wally R. Smith**, M.D., and **Thokozeni Lipato**, M.D., from the **adult sickle cell program at VCU Health** while participating in the clinical trial.

Safo spoke with VCU Health News about the new treatments as well as other therapies in development to help give patients a new life.

What causes sickle cell disease, and how does the disorder impact a person's daily life?

Sickle cell disease occurs through a genetic mutation that creates an altered form of hemoglobin, the protein that helps red blood cells deliver oxygen throughout the body. The defect causes hemoglobin to form into clusters which causes the red blood cells to become distorted into "sickle" shapes.

Normally, red blood cells are disc-shaped and flexible enough to flow easily through blood vessels. However, sickle-shaped red blood cells are rigid, making them more susceptible to damage as they circulate through the bloodstream, sometimes even becoming lodged or stuck in small blood vessels. This causes people to experience inflammation and excruciating periods of pain, called pain crises, on a nearly daily basis. Patients with sickle cell disease also have an increased risk for other health problems, such as severe anemia, stroke, infections and progressive organ damage. The symptoms can cause individuals to spend days or weeks in the hospital and have reduced life expectancy.

What has been the standard treatment for sickle cell disease?

In the past 30 years, there have been four FDA-approved drugs to treat sickle cell disease.

- Hydroxyurea induces fetal hemoglobin production to help prevent red blood cells from becoming sickle-shaped.
- L-glutamine (Endari) reduces oxidative stress, which can help red blood cells regain their flexibility as they travel through blood vessels.
- Crizanlizumab (Adakveo) prevents blood cells from sticking to the inner lining of blood vessels.
- Voxelotor (Oxbryta) increases hemoglobin's affinity for oxygen to stop the proteins from aggregating, consequently preventing red blood cells from becoming misshapen.

However, these approaches have only had a modest impact on treating symptoms and patients still experience harsh conditions as a result of this disease. Additionally, patients may receive blood transfusions to replace defective sickle hemoglobin, penicillin and pneumonia vaccines to reduce the risk of infections, as well as opioids to alleviate sickle cell pain crises.

More advanced measures can be taken to reduce or even eliminate the effects of sickle cell disease. For example, stem cell transplantation can be a cure for patients with the most severe forms of the disease. However, to go through with the procedure, a sibling of the patient needs to match as the stem cell donor, and patients can suffer from critical side effects, such as transplant rejection and even death.

patients can suffer from critical side effects, such as transplant rejection and even death.

How is CRISPR/Cas9 technology being used to treat sickle cell disease?

Gene therapy with CRISPR/Cas9 involves cutting a piece of DNA in bone marrow stem cells from the patient. The altered stem cells are infused back into the patient, which then begins to produce another form of hemoglobin, fetal hemoglobin, that does not clump together or make red blood cells sickle.

This form of gene-correction treatment may be suitable for patients with very severe cases of sickle cell disease, but it still requires a stem cell transplant to ensure the gene-modified cells can attach and multiply in the bone marrow and take the place of a significant portion of the bone marrow cells.

What are some of the barriers to access that still need to be addressed for gene therapy?

There are concerns about the price of the therapy, which is expected to cost upwards of $3 million per patient, which is beyond what many families can afford out-of-pocket. The treatment is also a very complex process, requiring frequent and extended stays in the hospital.

You and your colleagues at the VCU School of Pharmacy have been involved in developing a treatment option for sickle cell disease, called ILX-002. Can you explain how this medication could be used to treat this illness? And what is the status of this research project?

For the past three decades, our group at the VCU School of Pharmacy, and collaborators from the Children Hospital of Philadelphia, King Abdulaziz University in Saudi Arabia, and Illexcor Therapeutics, has been involved in the development of therapeutic agents to treat sickle cell disease. The over-arching goal is to develop a pill that can be taken once per day that allows individuals to have long-lasting lives that are largely free from the adverse effects of the disease.

Our focus has been finding a way to prevent sickle hemoglobin from forming clusters, which in turn could mitigate the downstream effects of the disease. Our latest drug candidate ILX-002 can directly bind to sickle hemoglobin and interfere with the aggregation process, and it is the first such drug to have this effect.

This type of treatment is the holy grail for sickle cell disease because there are simply too many people throughout the world with this condition to treat with gene-editing. Furthermore, if we can modify the disease significantly with a safe and effective pill, then more involved procedures would hopefully only be needed by individuals with the most severe cases of the disease. We anticipate that ILX-002 will begin human clinical trials in early 2025.

Learn more about the VCU Health patient involved in the sickle cell gene therapy clinical trial

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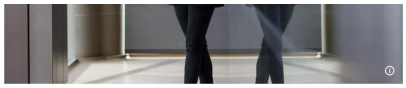
This gene therapy company is testing new tech to 'switch off' diabetes and obesity with a pill



By Natalie Huet

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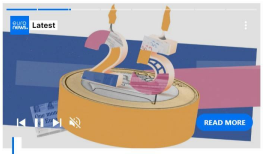
MeiraGTx is developing gene therapy with a "switch" that could revolutionise the treatment of diabetes, obesity and other complex diseases.

What if people who need regular injections to treat chronic diseases could instead take a pill that precisely controls the production of the right proteins and hormones inside their bodies?

A promising new gene therapy technology that aims to turn the human body into such a medicine-making factory could, if successful, push the boundaries of medicine and make certain treatments much more convenient and potentially less expensive.

MeiraGTx, a gene therapy company, is working to make this futuristic vision a reality.



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The British company already develops "traditional" gene therapy, which replaces missing or broken genes in people with inherited disorders. That side of business is booming, and the firm just opened a manufacturing facility in Ireland that could employ up to 300 people.

But MeiraGTx is also making strides in what it calls gene "regulation" therapy, which it says could help control much more precisely the genes that instruct cells to make – or stop making – certain proteins.

It's no easy feat and the technology would take years to bring to market, but it has the potential to make gene therapy even more life-changing for patients.

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"When you put a gene or replace a missing gene into a cell today, you put the gene in and it is expressed for the rest of that cell's life," MeiraGTx CEO Alexandria Forbes told Euronews Next.

"It's very hard to build a gene therapy that is switched on and off when it's needed, particularly in a disease. And what's even harder is to create a gene therapy which is switched on or off when you, the doctor or patient, want it to be".

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MeiraGTx says it has developed a switch of this sort that could help make patients' lives much easier: rather than injecting synthetic hormones and proteins into them, it could insert the gene that tells their body to make those, while a pill activates the gene only when the specific protein or hormone is needed.

Making the body make the drug

Take Epogen (epoetin alfa) – a well-known injectable drug that helps create more red blood cells when you're anaemic, with kidney disease or you're being treated for cancer.

"What you can do, for example, is put the gene for Epogen into the body, into the muscle, and have a switching system that only allows your body to make the natural form of Epogen when you take a pill," Forbes explained.

"So we don't have to make unnatural forms of these drugs – because what we're doing is we're providing the body with the message to make the drug, and that message is only switched on when we give the body a pill".

MeiraGTx told Euronews Next it has already tested this technology in animals and is hoping to start trialling it on humans in 2023.

If successful, it could have "huge, broad-ranging implications," Forbes said.

"This isn't only for gene therapy. It allows you to control cell therapy, immuno-oncology, antibody production – anything that is a protein or peptide that can be made by the body".

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How gene therapy works

Many traditional drugs involve making a protein outside the body, like insulin to fight diabetes or antibodies to fight cancer. That protein is manufactured in cells or bacteria outside the body and then is injected into the body on a regular basis as a treatment.

Gene therapy, by contrast, involves putting into the body a gene encoding the therapeutic protein: rather than injecting the protein over and over and over again, you put the gene for the protein into the person and the protein is made in the person's body.

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Gene therapies are typically used against inherited diseases, where a gene is missing or not functioning well. Gene therapy inserts into a patient a perfect copy of that gene to replace the missing or "broken" one.

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"So our drugs are actually genes - DNA - and they're delivered into the body by being encapsulated in viral proteins which act like a little spaceship and insert those genes that we've made into the appropriate cell," Forbes said.

This type of technology requires a very specific manufacturing process to ensure through rigorous testing that every single batch of these genes always has the same identical quality, safety and potency, she explained.

MeiraGTx controls this manufacturing in-house and has just inaugurated a new commercial-scale facility in Shannon, Ireland, that's set to employ 100 people initially and up to 300 as business grows.

MeiraGTx formally unveiled its new gene therapy manufacturing facility in Shannon, Ireland, on September 16, 2022, with Irish Prime Minister Micheál Martin in attendance - Courtesy MeiraGTx

Fighting obesity with a pill?

The company hopes the new site will help accelerate its development and delivery of gene therapy treatments to patients with an initial focus on rare inherited disorders affecting the eye, central nervous system, and salivary gland.

But MeiraGTx argues that adding a switch to be able to fine-tune gene therapy has the potential to considerably expand this range to also tackle non-hereditary diseases that affect hundreds of millions of people worldwide, including heart disease, cancer and diabetes.

It says it could even help fight **obesity**, arguably one of the biggest global health challenges.

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The causes behind obesity are complex and multi-faceted, genetic factors mean some people are more at risk than others, and the hormones that control appetite are very unstable and short-lasting.

A class of injectable diabetes drugs currently proving highly effective against obesity are GLP- 1 drugs, which help control blood sugar levels. But they work better in combination with several other gut peptides that affect metabolism.

The challenge, once again, is to precisely control the levels of these peptides.

MeiraGTx claims its technology may someday allow those hoping to lose weight to "switch on" the combination of genes that produce the hormones and peptides controlling their appetite, blood sugar levels and ultimately their fat.

"We can now put the genes for three natural gut peptides that control metabolism into the body and give a pill when we want those drugs," Forbes said.

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In theory, if clinical trials go well, the potential applications for other diseases are dizzying – and they directly raise the question of extending human life expectancy. But that should not be the priority right now, Forbes said.

"I think that currently we have really big problems with obesity, with Alzheimer's, with ways of living that mean we are young and living poorly," she said.

"And these sorts of products can be used to help address those really large indications, not just the rare gene replacements".

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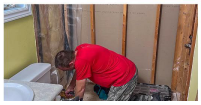

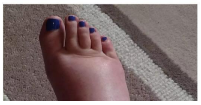

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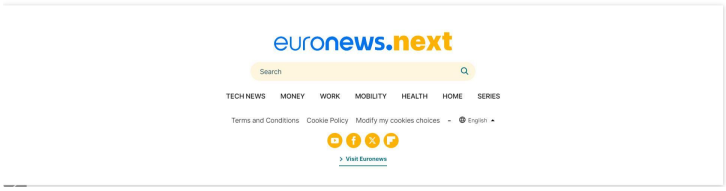
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Gene Therapy: Genes As Medicine

Gene therapy is the next generation of medicine targeting the underlying cause of a genetic disease. It has the potential to offer patients a transformational clinical benefit and dramatically improve quality of life.

What is a Gene? +

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Gene therapy is a new generation of medicine where a functioning gene is delivered to a targeted tissue in the body to produce a missing or nonfunctioning protein. By using genes as medicine, the underlying cause of a disease can be targeted at the cellular level, potentially with just one treatment.[5,6,7]

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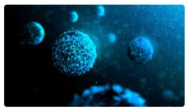

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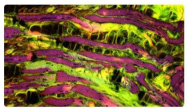

What Is Duchenne Muscular Dystrophy?

Muscular dystrophies are a debilitating group of illnesses that not only rob children of their ability to move properly, but can dramatically shorten their lives.

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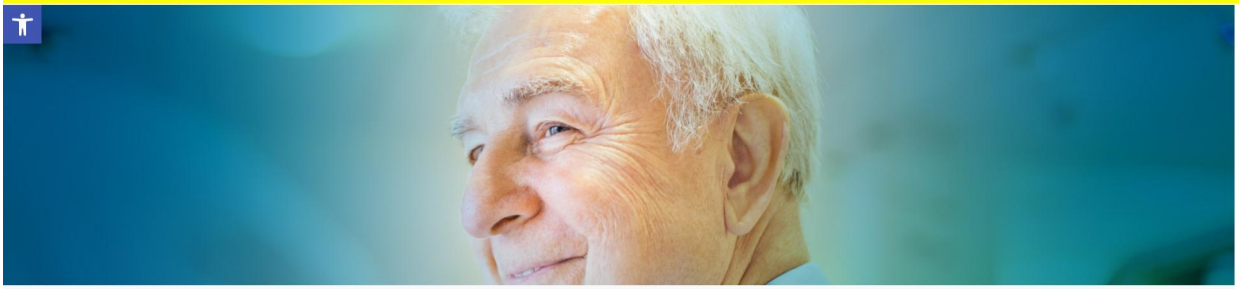

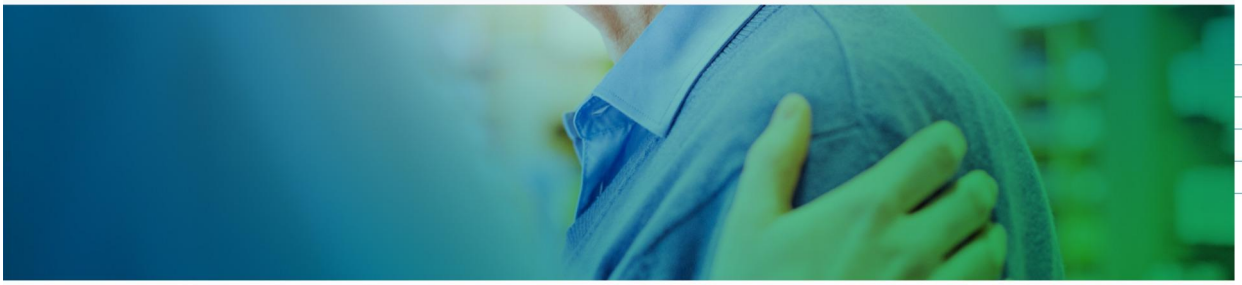

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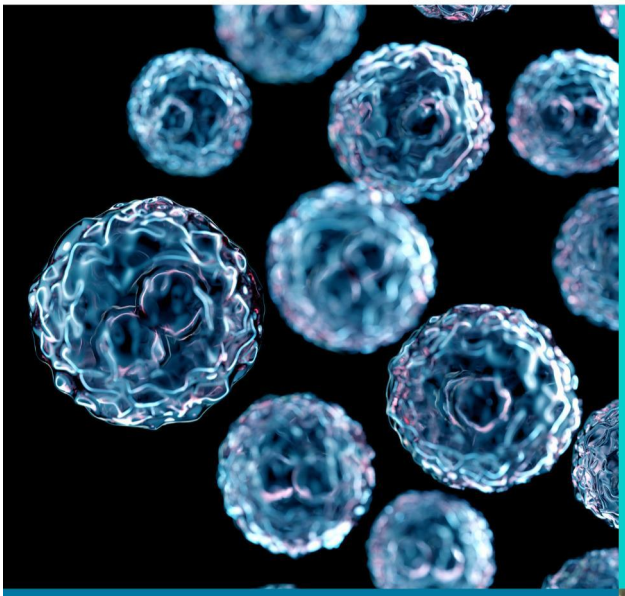

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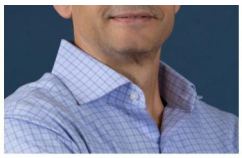

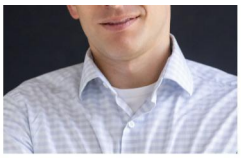

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Takeda, enGene Collaborate on Gene Pill

January 12, 2016

In a strategic alliance announced today, Takeda Pharmaceutical and enGene will begin work on a delivery system they term the "gene pill," a novel therapy for the treatment of gastrointestinal (GI) disease. The pair will work on discovery, development, and commercialization, leveraging enGene's non-viral vector platform in research involving

two Takeda-selected targets.

After preclinical proof of concept and IND-enabling studies, Takeda will have the option to license global rights for any promising candidates and then be responsible for further development and commercialization.

For its part, enGene will receive upfront payments of an undisclosed amount as well as reimbursement for research and development costs accrued. It will also be eligible to earn milestone payments based on meeting certain specific research, clinical, regulatory, and commercial goals, with royalties promised on the back end.

The companies also intend to pair on an oral antibody gene pill platform, with Takeda having options on up to three targets. The privately held enGene has been working on these gene pill delivery systems, which are designed to take effect on mucosal cells lining the gut, a concept that would include oral and enema treatment to localize delivery of immune-modulating proteins that could be used to treat a range of immune disorders.

This agreement is one of several that Takeda has entered into in recent months for the development of GI and gene therapies. enGene also partners with Janssen in development of therapies for inflammatory bowel disease.

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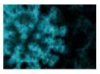

Amgen Opts-in on Generate:Biomedicines Collaboration for Drug Development

Read the Digital Edition

CLINICAL DATA

Biotech upstart enGene preps first trial for next-gen 'gene pill' tech

By **John Carroll** · Jan 26, 2015 10:09am

Montreal-based enGene has lined up $11.5 million in venture financing to put its next-gen approach to an inflammatory bowel disease treatment through its first clinical test.

The biotech has been at work developing a biopolymer-based DNA and RNAi delivery technology that can be used to deposit an oral treatment directly into the intestine, with an eye to "turning the gut into a protein factory." Its lead program for EG-12 is targeted at replacing currently used injectable drugs for IBD with a new approach that promises to be far more effective. And it claims that the same technology could be used to develop "gene pills" that can orally deliver protein therapies for the blockbuster diabetes market.

Forbion Capital Partners led the round, with new investors Québec's Fonds de solidarité FTQ and Pharmstandard International coming in. Existing investor Lumira Capital--via its Merck Lumira Biosciences Fund, which led the similar-sized Series A round in 2013--also participated. The new money will go to a Phase I/II study that aims to demonstrate the technology's ability to spur the gut-localized expression of the anti-inflammatory cytokine IL-10.

EnGene CEO Anthony Cheung tells FierceBiotech that the company started out trying to get genes into the gut, initially turning to viruses--as used by other gene therapy companies. This new polymer-based delivery tech gets the genes that encode proteins into the gut orally, where it breaks down and delivers the targeted therapeutic payload. If it works, this new tactic can bypass the kind of systemic toxicity that an injectable approach can trigger, as Schering-Plough experienced with its work on IL-10. And it promises to be the kind of therapy that can be easier to manufacture.

Cheung is also planning to take this technology and use it to develop a gene pill that can offer an oral approach to developing a long-acting insulin.

Right now enGene has 10 full-timers on the staff, which Cheung says will now swell to 30. And the money should be enough to get through Phase I/II while laying the groundwork for their insulin program.

Dr. Sander van Deventer

"While there has historically been a huge interest in the delivery of RNA in all its forms, nonviral delivery of DNA has surprisingly received little attention," commented Dr. Sander van Deventer, the managing partner of Forbion. "The unique technology of enGene effectively turns the gut into a protein factory, and given the strong genetic relation between IL-10 and ulcerative colitis, we expect EG-12 to provide significant benefit to IBD patients. Moreover, as the technology seems very well suited for oral formulation there is the potential for what is called a 'gene pill.' This novel technology could be used to treat not only IBD, but a much wider range of diseases, including those that are currently treated by parenteral protein replacement therapy. Forbion is very excited to have led this financing round."

- here's the release

ATTEND EVENTS

09-10 JAN
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Virtual Event

11-12 MAR
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