

# REGENERON PHARMACEUTICALS INC

## FORM 8-K (Current report filing)

Filed 01/16/97 for the Period Ending 01/14/97

Address	777 OLD SAW MILL RIVER RD TARRYTOWN, NY 10591-6707
Telephone	9143477000
CIK	0000872589
Symbol	REGN
SIC Code	2834 - Pharmaceutical Preparations
Industry	Biotechnology & Drugs
Sector	Healthcare
Fiscal Year	12/31

**SECURITIES AND EXCHANGE COMMISSION**  
Washington, D.C. 20549

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**FORM 8-K**

**CURRENT REPORT**

**PURSUANT TO SECTION 13 OR 15(d) OF THE  
SECURITIES EXCHANGE ACT OF 1934**

**Date of Report (Date of earliest event reported) January 14, 1997**

**REGENERON PHARMACEUTICALS, INC.**

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(Exact name of registrant as specified in its charter)

NEW YORK	0-19034	No. 13-3444607
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(State or other jurisdiction of incorporation)	(Commission File Number)	(IRS Employer Identification No.)

**777 OLD SAW MILL RIVER ROAD, TARRYTOWN, NY 10591-6707**  
(Address of principal executive offices) (Zip Code)

Registrant's telephone number, including area code (914) 347-7000

NOT APPLICABLE

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(Former name or former address, if changed since last report)

## **INFORMATION TO BE INCLUDED IN REPORT**

### **Item 5. Other Events.**

On January 10, 1997 and January 14, 1997, the Company issued press releases, copies of which are included as exhibits to this filing.

### **Item 7. Financial Statements and Exhibits.**

(c) Exhibits

99(a) Press Release dated January 10, 1997.

99(b) Press Release dated January 14, 1997.

**SIGNATURE**

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

**Regeneron Pharmaceuticals, Inc.**

By: /s/ Murray A. Goldberg

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*Murray A. Goldberg  
Vice President, Finance &  
Administration, Chief Financial  
Officer, and Treasurer*

*Date: January 14, 1997*

**Exhibit 99(a)**  
**BDNF PHASE 3 TRIAL**  
**DOES NOT DEMONSTRATE EFFICACY**

**FOR IMMEDIATE RELEASE**

THOUSAND OAKS, Calif., January 10, 1997 -- Amgen (NASDAQ:AMGEN) and Regeneron Pharmaceuticals, Inc. (NASDAQ:REGN) today announced that the Phase 3 clinical trial of Brain-Derived Neurotrophic Factor (BDNF) did not demonstrate clinical efficacy in patients with amyotrophic lateral sclerosis (ALS), commonly known as Lou Gehrig's Disease.

While the trial confirmed the safety and tolerability seen in earlier trials, it showed no statistically significant or clinically relevant difference in breathing capacity or survival between treatment and placebo groups. Breathing capacity was measured by forced vital capacity (FVC), a reliable measure of respiratory function and an established clinical indicator in this neurodegenerative disorder.

The trial was designed to evaluate effects of subcutaneous delivery of BDNF for ALS. Small, early-stage clinical trials investigating intrathecal administration for ALS and subcutaneous delivery for diabetic neuropathy are in progress and will continue. However, no further development of subcutaneous delivery for ALS is planned.

"Obviously, we are disappointed with these results," said Gordon Binder, chairman and chief executive officer of Amgen. "We had hoped that this trial would show benefit for patients with this tragic disease. We thank the patients and health care professionals who participated in the trial."

Leonard S. Schleifer, M.D., Ph.D., president and chief executive officer of Regeneron, also stressed his great disappointment. "Nevertheless," Schleifer said, "we continue to believe that neurotrophic factors have potential to benefit patients with neurological diseases. Thus, we will continue our pursuit of intrathecal use of BDNF in patients with ALS and both BDNF and NT-3 for treatment of peripheral neuropathies."

Amgen is a global biotechnology company that discovers, develops, manufactures and markets human therapeutics based on advances in cellular and molecular biology.

Regeneron (NASDAQ:REGN), based in Tarrytown, N.Y., is a leader in the application of molecular and cell biology to discover novel potential therapies for human medical conditions. The Company is applying its technological expertise in protein growth factors, their receptors and their mechanisms of action to the discovery and development of neurotrophic factors for the potential treatment of neurodegenerative diseases, peripheral neuropathies and nerve injury. More recently, Regeneron has used its technological expertise to attempt to identify treatments for diseases and conditions outside of the nervous system, such as inflammatory and muscle diseases, angiogenesis, hematopoiesis, and cancer.

Amgen and Regeneron are jointly developing two neurotrophic factors, BDNF and NT-3.

CONTACT: David Kaye, Amgen, 805/447-6692 (media) Denise Powell, Amgen, 805/447-4346 (investors) Murray Goldberg, Regeneron, 914/345-7492 (media/investors)

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NOTE: For additional information, health care professionals and patients can contact Amgen at 1-800-772-6436 or the ALS Association at 1-800-782-4747.

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**FOR IMMEDIATE RELEASE**

**REGENERON ANNOUNCES CLINICAL, SCIENTIFIC, FINANCIAL PLANS FOR THE FUTURE**

Focus on Current Clinical Trials, Rich Pipeline, Over \$95 Million in Cash

TARRYTOWN, New York (January 14, 1997) -- P. Roy Vagelos, M.D., Chairman of the Board of Regeneron Pharmaceuticals, Inc. (NASDAQ: REGN), in remarks to the investment community focusing on the five current and planned clinical trials of Regeneron products, its rich preclinical pipeline, and the financial soundness of the Company, described his vision of Regeneron's future. Dr. Vagelos also discussed the recent announcement that a Phase 3 clinical trial of subcutaneous delivery of brain-derived neurotrophic factor (BDNF) for the treatment of amyotrophic lateral sclerosis (ALS, commonly known as Lou Gehrig's disease) failed to achieve its primary endpoints.

The failure of BDNF is "obviously disappointing" not only to the Company, said Dr. Vagelos, but to the ALS patients and their families, to the clinicians who conducted the study, and to Amgen Inc., which sponsored the trial on behalf of Amgen-Regeneron Partners, a partnership equally owned by Amgen and Regeneron. The clinical trial data were not described, but will be presented at an appropriate scientific forum after the investigators and Amgen and Regeneron have completed their review and analysis.

The Phase 3 BDNF trial was initiated based on data from a Phase 2 trial and preclinical data. These data were not a "sure thing," Dr. Vagelos observed, but in light of the desperate nature of the disease and the information then available, "we continue to believe that the decision to move forward into the Phase 3 study was correct."

"On the other hand," said Dr. Vagelos, "because of the risks of the trial, Regeneron adopted a four-part plan to sustain the Company and advance its other clinical and preclinical programs even if the study did fail." The plan was:

- o Bring to the clinic BDNF, neurotrophin-3 (NT-3), and other neurotrophic factors for a variety of conditions of reasonable market size that were supported by preclinical findings;
- o Encourage Regeneron scientists to apply their technological expertise to areas beyond neurotrophic factors;
- o Expand vigorously discovery and development programs aimed at small molecule, orally active drugs; and
- o Build financial resources that could fund the Company's operations for two to three years.

Dr. Vagelos described the Company's current neurotrophic factor clinical program as including three BDNF trials and two trials of NT-3. BDNF is being tested by Amgen on behalf of Amgen-Regeneron Partners as a treatment for ALS, delivered intrathecally (directly into the spinal fluid), a route of delivery that may yield better results than subcutaneous delivery. Amgen is also testing BDNF in early stage, small European trials on Guillain Barre syndrome and diabetic neuropathy. Both these conditions involve peripheral neurons, which may allow for successful treatment via subcutaneous delivery. Amgen is conducting an early-stage NT-3 trial in diabetic neuropathy on behalf of Amgen-Regeneron Partners and plans to start a trial in chemotherapy-induced neuropathy during the first half of 1997. The Company anticipates announcing preliminary results of the NT-3 diabetic neuropathy study later in 1997.

In addition, Dr. Vagelos said, Regeneron is developing its second generation neurotrophic factor AXOKINE(R) for Huntington's disease and retinitis pigmentosa. A chemical lesion in animals similar to that found in Huntington's disease patients provides a model of the disease; AXOKINE has been shown to prevent the lesion in the model. In collaboration with Medtronic, Inc., Regeneron hopes to duplicate and extend these results and develop appropriate delivery systems for AXOKINE to the central nervous system of humans. In retinitis pigmentosa, animal models that duplicate the human condition have been developed and, in collaboration with scientists at the University of California, Regeneron has shown beneficial effects of AXOKINE in these models. This genetic disease leads to blindness and affects approximately 100,000 people in the United States. "We are working diligently to move AXOKINE into the clinic for this indication," said Dr. Vagelos, although there are significant preclinical hurdles that must be cleared before any clinical study could begin.

"Regeneron's scientists have led Regeneron and the world into several new preclinical fields," said Dr. Vagelos. Most recently, Dr. George Yancopoulos (Regeneron's Vice President, Discovery) and his team "broke open" the family of molecules they dub the Angiopoietins, which Cell, the leading molecular biology journal recently featured on its cover with three articles and a review. The Angiopoietins, which were identified and cloned by Regeneron after a significant international scientific race, may have a number of therapeutic applications. Said Dr. Vagelos: "We have discovered a family of Angiopoietins that includes both naturally occurring agonists and at least one antagonist. Thus, they might be used as agonists to stimulate blood vessel growth in situations of ischemia, where greater blood vessel flow is needed. As antagonists, they might inhibit blood vessel growth and thereby potentially inhibit cancer tumor growth. In addition, the receptors for these ligands are also found on hematopoietic stem cells, which may imply blood cell proliferation uses. We have a long (and exciting) way to go before we work out the biology and possible uses for this family of ligands and receptors."

Regeneron has also been a leader in the understanding of muscle atrophy, through its discovery of MuSK, a muscle-specific receptor that becomes widely expressed on the surface of muscle that has been injured or inactivated, a potentially significant unmet medical need. Dr. Yancopoulos and his team also identified agrin as the specific ligand for MuSK. Regeneron and The Procter & Gamble Company have entered into an exclusive worldwide agreement to discover and develop therapeutics for muscle diseases and disorders. Procter & Gamble invested \$10 million in the Company and will support \$3.75 million of annual research at Regeneron for up to five years.

The third preclinical program Dr. Vagelos described was Regeneron's cytokine traps, which are based on the Company's expertise in the surface receptors of cells. These proprietary proteins block the activity of a variety of cytokines, including IL-1, IL-4, and IL-6. They are very potent and comprised of human components. While the Company has not decided which trap to

develop first, Dr. Vagelos described the potential use of an IL-4 trap. Interleukin-4, or IL-4, is involved in the control or production of the immunoglobulin IGE that is involved in allergic diseases such as asthma. Reduction of IL-4 could reduce IGE, which could control the allergic reactions that lead to asthma attacks. If Regeneron's cytokine traps prove safe and effective, they could have an important medical role in a number of medical conditions.

Dr. Vagelos also discussed Noggin, a proprietary protein that recently has been shown to be a natural antagonist for certain bone morphogenetic proteins (BMPs). These BMPs initiate bone growth under normal conditions, such as skeletal growth and repair, but can also contribute to pathologically abnormal bone growth. For instance, spinal trauma or major burns are sometimes complicated by inappropriate formation of bone leading to major disability. Also, in approximately ten percent of hip replacement surgery, abnormal bone formation is a major complication. BMPs are believed to be involved in such pathological or abnormal bone formation. "Noggin might prevent such abnormal bone formation and the attendant morbidity," said Dr. Vagelos.

"We believe that we could have two or three new product candidates in the clinic by the end of 1998," said Dr. Vagelos.

"Our strategy also includes a focus on the discovery and development of small molecule, orally active drugs, which can offer medical and patient advantages over protein therapeutics," said Dr. Vagelos. Regeneron's strategy is to combine the enormous potential in molecular biology of the Company's discovery team with the skills of companies having combinatorial chemistry and high-throughput screening capabilities. Regeneron has two established relationships with such companies: Glaxo-Wellcome plc and Pharmacoepia, Inc.

During 1996, Dr. Vagelos said, Regeneron worked to secure adequate financial resources to support the clinical and research pipeline. These efforts resulted in \$68 million of new equity investments from Regeneron's corporate partners Amgen, Medtronic, and Procter & Gamble. In addition, annual payments are expected from Procter & Gamble for research support and from Merck & Co., Inc. for contract manufacturing of an intermediate for an approved Merck pediatric vaccine. "We anticipate reporting that Regeneron had over \$95 million in cash and cash equivalents at the end of 1996. Based on our current plans and information, we believe that these funds will enable us to continue operations for two to three years. We will consider other funding opportunities as they may arise in the future," said Dr. Vagelos.

Dr. Vagelos noted that Regeneron was founded for, and has historically been dedicated to, the discovery and development of drugs for the treatment of neurodegenerative disease, peripheral neuropathy, and nerve injury. These conditions, he said, are among the most intractable in medicine. For the most part, their mechanisms of action are unknown, and no surrogate markers of progression or treatment are available to measure the potential impact of therapeutic agents. On the other hand, preclinical data has accumulated over the years that indicate that neurotrophic factors could provide treatments for many of these conditions.

Dr. Vagelos said he had been attracted to join Regeneron, just over two years ago, by the presence of a group of people that, he believed, included "among the most talented scientific leaders I have ever encountered in my years in the pharmaceutical industry," including Leonard S. Schleifer, M.D., Ph.D., President and CEO, George D. Yancopoulos, M.D., Ph.D., Vice President, Discovery, and Ronald M. Lindsay, Ph.D., Vice President, Neurobiology. "The entire management team is comprised of people I knew I could work with, and whom I would bet on for success. My experience during the past two years has reaffirmed that original rationale for my

becoming Chairman of Regeneron." The Company's progress in the neurotrophic factor field, in discovering protein-based drug candidates outside of neurology, in entering into significant new collaborations, and in achieving financial stability provide the foundation for an exciting and productive future, he concluded.

Regeneron is a leader in the application of molecular and cell biology to discover novel potential therapeutics for human medical conditions. The Company is applying its technological expertise in protein growth factors, their receptors, and their mechanisms of action to the discovery and development of neurotrophic factors for the potential treatment of neurodegenerative diseases, peripheral neuropathies, and nerve injury. More recently, Regeneron has used its technological expertise to attempt to identify treatments for diseases outside of the nervous system, such as inflammatory and muscle diseases, angiogenesis, hematopoiesis, and cancer.

This news release discusses historical information and includes forward looking statements about Regeneron's products, programs, finances, and business, all of which involve a number of risks and uncertainties, such as risks associated with preclinical and clinical development of drugs and biologics, determinations by regulatory and administrative governmental authorities, competitive factors, technological developments, the availability and cost of capital, the costs of developing, producing, and selling products, and other material risks. A more complete description of these risks can be found in Regeneron's Form 10-K for the year ended December 31, 1995 and current Form 10-Q, copies of which should be read before making any investment decision regarding Regeneron common stock.

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Regeneron's recent news releases can be obtained by dialing (800) 311-0841 for fax copies or by accessing the Internet at [www.businesswire.com](http://www.businesswire.com)

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