

TARGETED GENETICS CORP /WA/
Form 424B3
July 31, 2007

**Filed pursuant to Rule 424(b)(3)
Registration Statement No. 333-144495**

13,734,575 Shares

TARGETED GENETICS CORPORATION

Common Stock

This prospectus relates to an aggregate of 13,734,575 shares of our common stock which may be disposed of by the Selling Shareholders listed on page 19, or their transferees. The shares covered hereby consist of 6,699,793 shares of our common stock and warrants to purchase up to 7,034,782 shares of our common stock. The shares and warrants were acquired directly from us on June 27, 2007 in a private placement that was exempt from the registration requirements of the federal securities laws. We will not receive any of the proceeds from the sale of these shares by the Selling Shareholders, but we will receive proceeds from the exercise of warrants, if exercised for cash.

Our common stock is quoted on the NASDAQ Capital Market under the symbol "TGEN." On July 30, 2007, the last reported sale price of our common stock was \$1.67 per share.

The Selling Shareholders may dispose of the shares covered hereby on the NASDAQ Capital Market or otherwise. These dispositions may be at fixed prices, at prevailing market prices at the time of sale, at prices related to the prevailing market price, at varying prices determined at the time of sale, or at negotiated prices. We will pay certain of the expenses of this offering, estimated to be \$1.6 million.

You should read this prospectus carefully before you invest.

**Investing in this stock involves a high degree of risk.
See "Risk Factors" beginning on page 4.**

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

The date of this prospectus is July 30, 2007.

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You should rely only on the information provided or incorporated by reference in this prospectus. Neither we nor the Selling Shareholders have authorized anyone to provide you with additional or different information or representations. You should not assume that the information in this prospectus is accurate as of any date other than its date, regardless of the time of delivery of this prospectus or any sale of common stock.

This prospectus is an offer to sell and a solicitation of an offer to buy the securities offered by this prospectus only in jurisdictions where the offer or sale is permitted.

In this prospectus, “Targeted Genetics,” “we,” “us” and “our” refer to Targeted Genetics Corporation and its subsidiaries. References to the “Securities Act,” refer to the Securities Act of 1933, as amended.

Prospectus Summary

The following summary is qualified in its entirety by, and should be read in conjunction with, the more detailed information appearing elsewhere in this prospectus and incorporated by reference herein. Before you decide to invest in our common stock, you should read the entire prospectus carefully.

About This Prospectus

This prospectus is part of a registration statement on Form S-3 filed by us with the Securities and Exchange Commission, or SEC, to register 13,734,575 shares of our common stock, consisting of 6,699,793 shares of common stock currently issued and outstanding, or the Common Shares, as well as up to 7,034,782 shares of common stock, or the Warrant Shares, issuable upon exercise of warrants, or the Warrants. Together the Common Shares and the Warrant Shares are referred to in this prospectus as the “Shares.” The Common Shares and Warrants were sold in connection with our private placement, which closed on June 27, 2007, as described in Current Reports on Form 8-K filed by us with the SEC on June 25, 2007 and June 28, 2007. The Shares are being registered for resale or other disposition by the Selling Shareholders or their transferees. We will not receive any proceeds from the sale or other disposition of the Shares registered hereunder, or interests therein. We will, however, receive proceeds from the exercise of any Warrants, if the exercise price is paid in cash. If all of the Warrants are exercised for cash, we will receive proceeds of approximately \$22.9 million, which we currently intend to use for general corporate purposes.

About Targeted Genetics Corporation

This summary does not contain all the information about us that may be important to you. You should read the more detailed information and consolidated financial statements and related notes that are incorporated by reference and are considered to be a part of this prospectus.

We are a clinical-stage biotechnology company focused on the development of targeted molecular therapies for the prevention and treatment of acquired and inherited diseases with unmet medical need. Our product development efforts target inflammatory arthritis, AIDS prophylaxis, congestive heart failure and Huntington's disease.

We develop gene therapy products and technologies for treating both acquired and inherited diseases. Our gene therapy product candidates are designed to treat disease by appropriately modifying cellular function at a genetic level. This involves introducing genetic material into target cells and expressing it in a manner that provides the desired effect. We have assembled a broad base of proprietary intellectual property that we believe gives us the potential to address the significant diseases that are the primary focus of our business. Our proprietary intellectual property includes gene therapy uses of certain genes, methods of transferring genetic material into cells, processes to manufacture our AAV-based product candidates and other proprietary technologies and processes. In addition, we have established expertise and development capabilities focused in the areas of preclinical research and development, manufacturing and manufacturing process scale-up, quality control, quality assurance, regulatory affairs and clinical trial design and implementation.

We have three product candidates in clinical trials. The first, tgAAC94, is an AAV-based product candidate being developed for the treatment of inflammatory arthritis. The second is an AAV-based prophylactic vaccine candidate for high-risk populations in developing nations to protect against HIV/AIDS. We are developing this program in collaboration with the International AIDS Vaccine Initiative, or IAVI, a non-profit organization, the Columbus Children's Research Institute at Children's Hospital in Columbus, Ohio, or CCRI, and The Children's Hospital of Philadelphia, or CHOP. The National Institute of Allergy and Infectious Disease, or NIAID, has awarded a \$21.75 million contract to us and our scientific collaborators at CHOP and CCRI. We have a subcontract with CHOP to complete work related to the NIAID contract, under which we may receive up to \$18.2 million over the five-year period of the contract. As of December 31, 2006, we had earned approximately \$1.5 million under this subcontract.

The purpose of the NIAID award is to develop AAV-based vaccines against HIV strains most prevalent in North America and Europe. The third product candidate in clinical trials, MYDICAR, utilizes an AAV vector to deliver the SERCA2a gene to heart muscle tissue for the treatment of congestive heart failure. We are developing this product candidate with Celladon Corporation, or Celladon.

We are also partnered with Sirna Therapeutics, Inc., or Sirna, a wholly owned subsidiary of Merck & Co., Inc., in a collaboration formed in January 2005, to develop short interfering RNA, AAV-based therapies for the treatment of Huntington's disease.

The development of pharmaceutical products, including our potential inflammatory arthritis, prophylactic AIDS vaccine, congestive heart failure, and Huntington's disease product candidates discussed above, involves extensive preclinical development followed by human clinical trials that take several years or more to complete. The length of time required to completely develop any product candidate varies substantially according to the type, complexity and novelty of the product candidate, the degree of involvement by a development partner and the intended use of the product candidate. Our commencement and rate of completion of clinical trials may vary or be delayed for many reasons, including those discussed in the section entitled "Risk Factors" presented below.

We were incorporated in the state of Washington in 1989. Our executive offices are located at 1100 Olive Way, Suite 100, Seattle, Washington 98101, and our telephone number is (206) 623-7612.

For more information about us, you should read this prospectus, including the information described in the section of this prospectus entitled "Where You Can Find More Information," together with our consolidated financial statements and related notes.

RISK FACTORS

Our business faces significant risks. You should carefully consider the following risk factors, in addition to the other information included or incorporated by reference in this prospectus, before purchasing our securities. These risks may not be the only risks we face. Additional risks that we do not yet know of or that we currently think are immaterial also may impair our business. You could lose all or part of your investment if any of the following risks actually occurs.

Risks Related to Our Business

We expect to continue to operate at a loss and may never become profitable.

Substantially all of our revenue to date has been derived under collaborative research and development agreements relating to the development of our potential product candidates. We have incurred, and will continue to incur for the foreseeable future, significant expense to develop our research and development programs, conduct preclinical studies and clinical trials, seek regulatory approval for our product candidates and provide general and administrative support for these activities. As a result, we have incurred significant net losses since inception, and we expect to continue to incur substantial additional losses in the future. As of March 31, 2007, we had an accumulated deficit of \$287.9 million. We may never be able to commercialize our products or generate profits and, if we do become profitable, we may be unable to sustain or increase profitability.

All of our product candidates are in early-stage clinical trials or preclinical development, and if we are unable to successfully develop and commercialize our product candidates we will be unable to generate sufficient capital to maintain our business.

In March 2006, we initiated a Phase I/II trial for our inflammatory arthritis product candidate in the United States and Canada. We will not generate any product revenue for at least several years and then only if we can successfully develop and commercialize our product candidates. Commercializing our potential products depends on successful completion of additional research and development and testing, in both preclinical development and clinical trials. Clinical trials may take several years or more to complete. The commencement, cost and rate of completion of our clinical trials may vary or be delayed for many reasons. If we are unable to successfully complete preclinical and clinical development of some or all of our product candidates in a timely manner, we may be unable to generate

sufficient product revenue to maintain our business.

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Even if our potential products succeed in clinical trials and are approved for marketing, these products may never achieve market acceptance. If we are unsuccessful in commercializing our product candidates for any reason, including greater effectiveness or economic feasibility of competing products or treatments, the failure of the medical community or the public to accept or use any products based on gene delivery, inadequate marketing and distribution capabilities or other reasons discussed elsewhere in this section, we will be unable to generate sufficient product revenue to maintain our business.

The success of our clinical trials and preclinical studies may not be indicative of results in a large number of subjects of either safety or efficacy.

The successful results of our technology in preclinical studies using animal models may not be predictive of the results that we will see in our clinical trials. In addition, results in early-stage clinical trials are based on limited numbers of subjects and generally test for drug safety rather than efficacy. Our reported progress and results from our early phases of clinical testing of our product candidates may not be indicative of progress or results that will be achieved from larger populations, which could be less favorable. Moreover, we do not know if the favorable results we have achieved in clinical trials will have a lasting or repeatable effect. If a larger group of subjects does not experience positive results or if any favorable results do not demonstrate a beneficial effect, our product candidates that we advance to clinical trials may not receive approval from the FDA for further clinical trials or commercialization. For example, in March 2005, we discontinued the development of tgAAVCF, our product candidate for the treatment of cystic fibrosis, following the analysis of Phase II clinical trial data in which tgAAVCF failed to achieve the efficacy endpoints of the trial.

If we are unable to raise additional capital when needed, we will be unable to conduct our operations and develop our potential products.

Because internally generated cash flow will not fund development and commercialization of our product candidates, we will require substantial additional financial resources. Our future capital requirements will depend on many factors, including:

- the rate and extent of scientific progress in our research and development programs;
- the timing, costs and scope of, and our success in, conducting clinical trials, obtaining regulatory approvals and pursuing patent prosecutions;
- competing technological and market developments;
- the timing and costs of, and our success in, any product commercialization activities and facility expansions, if and as required; and
- the existence and outcome of any litigation or administrative proceedings involving intellectual property.

Additional sources of financing could involve one or more of the following:

- entering into additional product development collaborations;
- mergers and acquisitions;
- issuing equity in the public or private markets;
- extending or expanding our current collaborations;

- selling or licensing our technology or product candidates;
- borrowing under loan or equipment financing arrangements; and
- issuing debt.

Additional funding may not be available to us on reasonable terms, if at all. Our ability to issue equity, and our ability to issue it at the current market price, may be adversely affected by the fact that we are presently ineligible under SEC rules to utilize Form S-3 for primary offerings of our securities because the aggregate market value of our outstanding common stock held by non-affiliates is less than \$75.0 million.

The perceived risk associated with the possible sale of a large number of shares of our common stock could cause some of our shareholders to sell their stock, thus causing the price of our stock to decline. In addition, actual or anticipated downward pressure on our stock price due to actual or anticipated sales of stock could cause some institutions or individuals to engage in short sales of our common stock, which may itself cause the price of our stock to decline.

If our stock price declines, we may be unable to raise additional capital. A sustained inability to raise capital could force us to go out of business. Significant declines in the price of our common stock could also impair our ability to attract and retain qualified employees, reduce the liquidity of our common stock and result in the delisting of our common stock from the NASDAQ Capital Market.

The funding that we expect to receive from our collaborations depends on continued scientific progress under the collaborations and our collaborators' ability and willingness to continue or extend the collaboration. If we are unable to successfully access additional capital, we may need to scale back, delay or terminate one or more of our development programs, curtail capital expenditures or reduce other operating activities. We may also be required to relinquish some rights to our technology or product candidates or grant or take licenses on unfavorable terms, either of which would reduce the ultimate value to us of our technology or product candidates.

Failure to recruit subjects could delay or prevent clinical trials of our potential products, which could delay or prevent the development of potential products.

Identifying and qualifying subjects to participate in clinical trials of our potential products is critically important to our success. The timing of our clinical trials depends on the speed at which we can recruit subjects to participate in testing our product candidates. We have experienced delays in some of our clinical trials due to difficulty recruiting subjects, and we may experience similar delays in the future. If subjects are unwilling to participate in our gene therapy trials because of negative publicity from adverse events in the biotechnology or gene therapy industries or for other reasons, including competitive clinical trials for similar patient populations, the timeline for recruiting subjects, conducting trials and obtaining regulatory approval of potential products will be delayed. These delays could result in increased costs, delays in advancing our product development, delays in testing the effectiveness of our technology or termination of the clinical trials altogether.

The regulatory approval process for our product candidates is costly, time-consuming and subject to unpredictable changes and delays, and our product candidates may never receive regulatory approval.

No gene therapy products have received regulatory approval for marketing from the U.S. Food and Drug Administration, or FDA. Because our product candidates involve new and unproven technologies, we believe that the regulatory approval process may proceed more slowly compared to clinical trials involving traditional drugs. The

FDA and applicable state and foreign regulators must conclude at each stage of clinical testing that our clinical data suggest acceptable levels of safety in order for us to proceed to the next stage of clinical trials. In addition, gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the National Institute of Health, or NIH are subject to review by the NIH's Office of Biotechnology Activities Recombinant DNA Advisory Committee, or RAC. Although NIH guidelines do not have regulatory status, the RAC review process can impede the initiation of the trial, even if the FDA has reviewed the trial and approved its initiation. Moreover, before a clinical trial can begin at an NIH-funded institution, that institution's Institutional Biosafety Committee must review the proposed clinical trial to assess the safety of the trial.

The regulatory process for our product candidates is costly, time-consuming and subject to unpredictable delays. The clinical trial requirements of the FDA, NIH and other agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use of the potential products. In addition, regulatory requirements governing gene therapy products have changed frequently and may change in the future. Accordingly, we cannot predict how long it will take or how much it will cost to obtain regulatory approvals for clinical trials or for manufacturing or marketing our potential products. Some or all of our product candidates may never receive regulatory approval. A product candidate that appears promising at an early stage of research or development may not result in a commercially successful product. Our clinical trials may fail to demonstrate the safety and efficacy of a product candidate or a product candidate may generate unacceptable side effects or other problems during or after clinical trials. Should this occur, we may have to delay or discontinue development of the product candidate, and the partner, if any, that supports development of such product candidate may terminate its support. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market will decrease our ability to generate sufficient product revenue to maintain our business.

If we are unable to obtain or maintain licenses for necessary third-party technology on acceptable terms or to develop alternative technology, we may be unable to develop and commercialize our product candidates.

We have entered into exclusive and nonexclusive license agreements that give us and our partners rights to use technologies owned or licensed by commercial and academic organizations in the research, development and commercialization of our potential products. For example, we have a gene therapy technology license agreement with Amgen Inc., or Amgen, as the successor to Immunex Corporation, or Immunex, under which we have licensed rights to certain Immunex proprietary technology specifically applicable to gene therapy applications. In a February 2004 letter, Amgen took the position that we are not licensed, either exclusively or nonexclusively, to use Immunex intellectual property covering TNFR:Fc or therapeutic uses for TNFR:Fc. We have responded with a letter confirming our confidence that the gene therapy technology license agreement provides us with an exclusive worldwide license to use the gene construct coding for TNFR:Fc for gene therapy applications. We have had, and expect to have further, communications with Amgen regarding our differences. Notwithstanding our confidence, it is possible that a resolution of those differences, through litigation or otherwise, could cause delay or discontinuation of our development of tgAAC94 or our inability to commercialize any resulting product.

We believe that we will need to obtain additional licenses to use patents and unpatented technology owned or licensed by others for use, compositions, methods, processes to manufacture compositions, processes to manufacture and purify gene delivery product candidates and other technologies and processes for our present and potential product candidates. If we are unable to maintain our current licenses for third-party technology or obtain additional licenses on acceptable terms, we may be required to expend significant time and resources to develop or license replacement technology. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates. In addition, the license agreements for technology for which we hold exclusive licenses typically contain provisions that require us to meet minimum development milestones in order to maintain the license on an exclusive basis for some or all fields of the license. We also have license agreements for some of our technologies that may require us to sublicense certain of our rights. If we do not meet these requirements, our licensor may convert all or a portion of the license to a nonexclusive license or, in some cases, terminate the license.

In many cases, patent prosecution of our licensed technology is controlled solely by the licensor. If our licensors fail to obtain and maintain patent or other protection for the proprietary intellectual property we license from them, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, and our competitors could market competing products using the intellectual property. Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues and is complicated by the rapid pace of scientific discovery in our industry. Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

Litigation involving intellectual property, product liability or other claims and product recalls could strain our resources, subject us to significant liability, damage our reputation or result in the invalidation of our proprietary rights.

As our product development efforts progress, most particularly in potentially significant markets such as HIV/AIDS, congestive heart failure or inflammatory arthritis therapies, the risk increases that others may claim that our processes and product candidates infringe on their intellectual property rights. In addition, administrative proceedings, litigation or both may be necessary to enforce our intellectual property rights or determine the rights of others. Defending or pursuing these claims, regardless of their merit, would be costly and would likely divert management's attention and resources away from our operations. If there were to be an adverse outcome in litigation or an interference proceeding, we could face potential liability for significant damages or be required to obtain a license to the patented process or technology at issue, or both. If we are unable to obtain a license on acceptable terms, or to develop or obtain alternative technology or processes, we may be unable to manufacture or market any product or potential product that uses the affected process or technology.

Clinical trials and the marketing of any potential products may expose us to liability claims resulting from the testing or use of our products. Gene therapy treatments are new and unproven, and potential known and unknown side effects of gene therapy may be serious and potentially life-threatening. Product liability claims may be made by clinical trial participants, consumers, healthcare providers or other sellers or users of our products. Although we currently maintain liability insurance, the costs of product liability and other claims against us may exceed our insurance coverage. In addition, we may require increased liability coverage as additional product candidates are used in clinical trials or commercialized. Liability insurance is expensive and may not continue to be available on acceptable terms. A product liability or other claim or product recall not covered by or exceeding our insurance coverage could significantly harm our financial condition. In addition, adverse publicity resulting from a product recall or a liability claim against us, one of our partners or another gene therapy company could significantly harm our reputation and make it more difficult to obtain the funding and collaborative partnerships necessary to maintain our business.

We may be unable to adequately protect our proprietary rights domestically or overseas, which may limit our ability to successfully market any product candidates.

Our success depends substantially on our ability to protect our proprietary rights and operate without infringing on the proprietary rights of others. We own or license patents and patent applications, and will need to license additional patents, for genes, processes, practices and techniques critical to our present and potential product candidates. If we fail to obtain and maintain patent or other intellectual property protection for this technology, our competitors could

market competing products using those genes, processes, practices and techniques. The patent process takes several years and involves considerable expense. In addition, patent applications and patent positions in the field of biotechnology are highly uncertain and involve complex legal, scientific and factual questions. Our patent applications may not result in issued patents and the scope of any patent may be reduced both before and after the patent is issued. Even if we secure a patent, the patent may not provide significant protection and may be circumvented or invalidated.

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We also rely on unpatented proprietary technology and technology that we have licensed on a nonexclusive basis. While we take precautions to protect our proprietary unpatented technology, we may be unable to meaningfully protect this technology from unauthorized use or misappropriation by a third party. Our competitors could also obtain rights to our nonexclusively licensed proprietary technology. In any event, other companies may independently develop equivalent proprietary information and techniques. If our competitors develop and market competing products using our unpatented or nonexclusively licensed proprietary technology or substantially similar technology, our products, if successfully developed, could suffer a reduction in sales or be forced out of the market.

If we do not attract and retain qualified personnel, we may be unable to develop and commercialize some of our potential products.

Our future success depends in large part on our ability to attract and retain key technical and management personnel. All of our employees, including our executive officers, can terminate their employment with us at any time. We have programs in place designed to retain personnel, including competitive compensation packages and programs to create a positive work environment. Other companies, research and academic institutions and other organizations in our field compete intensely for employees, however, and we may be unable to retain our existing personnel or attract additional qualified employees and consultants. If we experience significant turnover or difficulty in recruiting new personnel, our research and development of product candidates could be delayed and we could experience difficulty in generating sufficient revenue to maintain our business.

If we lose our collaborative partners, we may be unable to develop our potential products.

A portion of our operating expenses are funded through our collaborative agreements with third parties. Our HIV/AIDS vaccine collaboration with CHOP and CCRI is funded through a subcontract with the NIAID, which is a U.S. government agency. We also have contracts with two biotechnology companies, Celladon and Sirna, and one public health organization, IAVI. Each of these collaborations provides for funding, collaborative development, intellectual property rights or expertise to develop certain of our product candidates. With limited exceptions, each collaborator has the right to terminate its obligation to provide research funding at any time for scientific or business reasons. In addition, to the extent that funding is provided by a collaborator for non-program-specific uses, the loss of significant amounts of collaborative funding could result in the delay, reduction or termination of additional research and development programs, a reduction in capital expenditures or business development and other operating activities, or any combination of these measures.

If our partners or scientific consultants terminate, reduce or delay our relationships with them, we may be unable to develop our potential products.

Our partners provide funding, manage regulatory filings, aid and augment our internal research and development efforts and provide access to important intellectual property and know-how. Their activities include, for example, support in processing the regulatory filings of our product candidates and funding clinical trials. Our outside scientific consultants and contractors perform research, develop technology and processes to advance and augment our internal efforts and provide access to important intellectual property and know-how. Their activities include, for example, clinical evaluation of our product candidates, product development activities performed under our research collaborations, research under sponsored research agreements and contract manufacturing services. Collaborations with established pharmaceutical and biotechnology companies and academic, research and public health organizations often provide a measure of validation of our product development efforts in the eyes of securities analysts, investors and the medical community. The development of certain of our potential products, and therefore the success of our business, depends on the performance of our partners, consultants and contractors. If they do not dedicate sufficient time, regulatory or other technical resources to the research and development programs for our product candidates or if they do not perform their obligations as expected, we may experience delays in, and may be unable to continue, the preclinical or clinical development of those product candidates. Each of our collaborations and scientific consulting relationships concludes at the end of the term specified in the applicable agreement unless we and our partners agree

to extend the relationship. Any of our partners may decline to extend the collaboration, or may be willing to extend the collaboration only with a significantly reduced scope. Competition for scientific consultants and partners in gene therapy is intense. We may be unable to successfully maintain our existing relationships or establish additional relationships necessary for the development of our product candidates on acceptable terms, if at all. If we are unable to do so, our research and development programs may be delayed or we may lose access to important intellectual property or know-how.

If we do not develop adequate development, manufacturing, sales, marketing and distribution capabilities, either alone or with our business partners, we will be unable to generate sufficient product revenue to maintain our business.

Our potential products require significant development of new processes and design for the advancement of the product candidate through manufacture, preclinical and clinical testing. We may be unable to continue development or meet critical milestones with our partners due to technical or scientific issues related to manufacturing or development. We currently do not have the physical capacity to manufacture large-scale quantities of our potential products. This could limit our ability to conduct large clinical trials of a product candidate and to commercially launch a successful product candidate. In order to manufacture product at such scale, we will need to expand or improve our current facilities and staff or supplement them through the use of contract providers. If we are unable to obtain and maintain the necessary manufacturing capabilities, either alone or through third parties, we will be unable to manufacture our potential products in quantities sufficient to sustain our business. Moreover, we are unlikely to become profitable if we, or our contract providers, are unable to manufacture our potential products in a cost-effective manner.

In addition, we have no experience in sales, marketing and distribution. To successfully commercialize any products that may result from our development programs, we will need to develop these capabilities, either on our own or with others. We intend to enter into collaborations with other entities to utilize their mature marketing and distribution capabilities, but we may be unable to enter into marketing and distribution agreements on favorable terms, if at all. If our current or future collaborative partners do not commit sufficient resources to timely marketing and distributing our future products, if any, and we are unable to develop the necessary marketing and distribution capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business.

Post-approval manufacturing or product problems or failure to satisfy applicable regulatory requirements could prevent or limit our ability to market our products.

Commercialization of any products will require continued compliance with FDA and other federal, state and local regulations. For example, our current manufacturing facility, which is designed for manufacturing our AAV vectors for clinical and development purposes, is subject to the Good Manufacturing Practices requirements and other regulations of the FDA, as well as to other federal, state and local regulations such as the Occupational Health and Safety Act, the Toxic Substances Control Act, the Resource Conservation and Recovery Act and the Environmental Protection Act. Any future manufacturing facility that we may construct for large-scale commercial production will also be subject to regulation. We may be unable to obtain regulatory approval for or maintain in operation any manufacturing facility. In addition, we may be unable to attain or maintain compliance with current or future regulations relating to manufacture, safety, handling, storage, record keeping or marketing of potential products. If we fail to comply with applicable regulatory requirements or discover previously unknown manufacturing, contamination, product side effects or other problems after we receive regulatory approval for a potential product, we may suffer restrictions on our ability to market the product or be required to withdraw the product from the market.

Risks Related to Our Industry

Adverse events in the field of gene therapy could damage public perception of our potential products and negatively affect governmental approval and regulation.

Public perception of our product candidates could be harmed by negative events in the field of gene transfer. Serious adverse events, including patient deaths, have occurred in clinical trials. Adverse events in our clinical trials and the resulting publicity, as well as any other adverse events in the field of gene therapy that may occur in the future, could result in a decrease in demand for any products that we may develop. The commercial success of our product candidates will depend in part on public acceptance of the use of gene therapy for preventing or treating human diseases. If public perception is influenced by claims that gene therapy is unsafe, our product candidates may not be accepted by the general public or the medical community. The public and the medical community may conclude that our technology is unsafe.

Future adverse events in gene therapy or the biotechnology industry could also result in greater governmental regulation, unfavorable public perception, stricter labeling requirements and potential regulatory delays in the testing or approval of our potential products. Any increased scrutiny could delay or increase the costs of our product development efforts or clinical trials.

The intense competition and rapid technological change in our market may result in failure of our potential products to achieve market acceptance.

We face increasingly intense competition from a number of commercial entities and institutions that are developing gene therapy technologies. Our competitors include early-stage and more established gene delivery companies, other biotechnology companies, pharmaceutical companies, universities, research institutions and government agencies developing gene therapy products or other biotechnology-based therapies designed to treat the diseases on which we focus. We also face competition from companies using more traditional approaches to treating human diseases, such as surgery, medical devices and pharmaceutical products. If our product candidates become commercial gene therapy products, they may affect commercial markets of the analogous protein or traditional pharmaceutical therapy. This may result in lawsuits, demands, threats or patent challenges by others in an effort to reduce our ability to compete. In addition, we compete with other companies to acquire products or technology from research institutions or universities. Many of our competitors have substantially more resources, including research and development personnel, capital and infrastructure, than we do. Many of our competitors also have greater experience and capabilities than we do in:

- research and development;
- clinical trials;
- obtaining FDA and other regulatory approvals;
- manufacturing; and
- marketing and distribution.

In addition, the competitive positions of other companies, institutions and organizations, including smaller competitors, may be strengthened through collaborative relationships. Consequently, our competitors may be able to develop, obtain patent protection for, obtain regulatory approval for, or commercialize new products more rapidly than we do, or manufacture and market competitive products more successfully than we do. This could limit the prices we could charge for the products that we are able to market or result in our products failing to achieve market acceptance.

Gene therapy is a rapidly evolving field and is expected to continue to undergo significant and rapid technological change and competition. Rapid technological development by our competitors, including development of technologies, products or processes that are more effective or more economically feasible than those we have developed, could result in our actual and proposed technologies, products or processes losing market share or becoming obsolete.

Healthcare reform measures and the unwillingness of third-party payors to provide adequate reimbursement for the cost of our products could impair our ability to successfully commercialize our potential products and become profitable.

Sales of medical products and treatments, both domestically and abroad, substantially depend on the availability of reimbursement to the consumer from third-party payors. Our potential products may not be considered cost-effective by third-party payors, who may not provide coverage at the price set for our products, if at all. If purchasers or users of our products are unable to obtain adequate reimbursement, they may forego or reduce their use of our products. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.

Increasing efforts by governmental and third-party payors, such as Medicare, private insurance plans and managed care organizations, to cap or reduce healthcare costs will affect our ability to commercialize our product candidates and become profitable. We believe that third-party payors will attempt to reduce healthcare costs by limiting both coverage and level of reimbursement for new products approved by the FDA. There have been and will continue to be a number of federal and state proposals to implement government controls on pricing, the adoption of which could affect our ability to successfully commercialize our product candidates. Even if the government does not adopt any such proposals or reforms, their announcement could impair our ability to raise capital.

Our use of hazardous materials exposes us to liability risks and regulatory limitations on their use, either of which could reduce our ability to generate product revenue.

Our research and development activities involve the controlled use of hazardous materials, including chemicals, biological materials and radioactive compounds. Our safety procedures for handling, storing and disposing of these materials must comply with federal, state and local laws and regulations, including, among others, those relating to solid and hazardous waste management, biohazard material handling, radiation and air pollution control. We may be required to incur significant costs in the future to comply with environmental or other applicable laws and regulations. In addition, we cannot eliminate the risk of accidental contamination or injury from hazardous materials. If a hazardous material accident were to occur, we could be held liable for any resulting damages, and this liability could exceed our insurance and financial resources. Accidents unrelated to our operations could cause federal, state or local regulatory agencies to restrict our access to hazardous materials needed in our research and development efforts, which could result in delays in our research and development programs. Paying damages or experiencing delays caused by restricted access could reduce our ability to generate revenue and make it more difficult to fund our operations.

Risks Related to Our Common Stock

If we sell additional shares, our stock price may decline as a result of the dilution that will occur to existing shareholders.

Until we are profitable, we will need significant additional funds to develop our business and sustain our operations. Any additional sales of shares of our common stock are likely to have a dilutive effect on our then-existing shareholders. Subsequent sales of these shares in the open market could also have the effect of lowering our stock price, thereby increasing the number of shares we may need to issue in the future to raise the same dollar amount and consequently further diluting our outstanding shares. These future sales could also have an adverse effect on the market price of our shares and could result in additional dilution to the holders of our shares.

The perceived risk associated with the possible sale of a large number of shares could cause some of our shareholders to sell their stock, thus causing the price of our stock to decline. In addition, actual or anticipated downward pressure on our stock price due to actual or anticipated sales of stock could cause some institutions or individuals to engage in short sales of our common stock, which may itself cause the price of our stock to decline.

If our stock price declines, we may be unable to raise additional capital. A sustained inability to raise capital could force us to go out of business. Significant declines in the price of our common stock could also impair our ability to attract and retain qualified employees, reduce the liquidity of our common stock and result in the delisting of our common stock from the NASDAQ Capital Market.

Concentration of ownership of our common stock may give certain shareholders significant influence over our business.

A small number of investors own a significant number of shares of our common stock. Prior to the closing of this private placement, Biogen Idec held approximately 2.2 million shares and Elan held approximately 1.2 million shares of our common stock. Together these holdings represented approximately 25% of our common shares outstanding as reported in our Form 10-Q filed on May 9, 2007. This concentration of stock ownership may allow these shareholders to exercise significant control over our strategic decisions and block, delay or substantially influence all matters requiring shareholder approval, such as:

- election of directors;

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