Your participation in the Treatment Development Program is an investment in aggressive, results-driven research.
Executive Summary

The mission of the National Alopecia Areata Foundation (NAAF) Treatment Development Program (TDP) is to find a safe and effective treatment or cure for alopecia areata to benefit the 145 million people worldwide who have, had or will develop alopecia areata in their lifetime.

Since 1985, based on targeted research aims set forth during our biennial research summits, NAAF has awarded 178 research grants totaling $4.4 million. Of that, 38 grants totaling $1.65 million have been directed to the line of inquiry that has led to the current clinical trials with JAK inhibitors that are showing such promise for re-growing hair in people with alopecia areata.

Bringing drugs from discovery to market approval takes an average of 15 years and costs over $1 billion. However, NAAF’s strategic investment of $1.65 million to support the development of a mouse model, the creation of a registry, and immunogenetic research which has led to the investigative trajectory that we now find so promising, have shortened that timeline considerably.

If the investigative trajectory that we now find so promising proves safe and durable, we could reasonably expect to see a treatment on the market for alopecia areata as early as 2020.

NAAF Invests Strategically in Research to Find a Treatment or a Cure for Alopecia Areata

NAAF’s first grant to support genetic research was awarded in 1988. In 1989, NAAF funded the first of ten immunogenetics grants. NAAF funded development of an alopecia areata mouse model with 14 grants starting in 1992. NAAF and the National Institutes of Health* funded the development and creation of the Alopecia Areata Registry, Biobank and Clinical Trials Network (Registry), and in 1997, NAAF began funding Dr. Angela Christiano’s genetic work (the first of 12 grants) that led to the Genome-Wide Association Study (GWAS) that was published in Nature in 2010.

In 2014, NAAF advisors and grantees Drs. Raphael Clynes, Angela Christiano and Julian MacKay-Wiggan made a major step forward in their research to find a cure for alopecia areata. Their exciting preliminary findings with a drug approved by the FDA for another indication showed near complete hair regrowth in several patients with moderate-to-severe alopecia areata. Their report of the incidental findings came out just before Dr. Christiano’s publication in the September 2014 issue of Nature Medicine, which included all of the underlying genetic, immunologic and animal testing research.

Dr. Brett King at Yale University attended Dr. Christiano’s poster presentation at a medical meeting in the spring of 2014 that showed the results of testing this same drug on the mouse model. Dr. King tested the drug on his patient who had both alopecia areata and the condition for which the drug was already FDA-approved. His report of the incidental findings came out just before Dr. Christiano’s publication in the September 2014 issue of Nature Medicine, which included all of the underlying genetic, immunologic and animal testing research.

The importance of the Registry is demonstrated by the valuable research of Dr. Christiano. She, along with with Drs. Clynes and Mackay-Wiggan, and other members of the Columbia University team, have been active participants in the NAAF Research Summits aimed at developing new treatments. Without quick and easy access to samples through the Registry—and the strategic framework of the TDP and associated Research Summits—these breakthroughs would never have been realized in only a few short years. This work is a prime example of NAAF’s strategic investment in research infrastructure. With the research underway, the Registry’s scientific significance extends beyond alopecia areata to other autoimmune diseases.

Over the next three years, NAAF seeks to grow the TDP in order to support this essential work and ensure that these new groundbreaking findings can further the necessary clinical testing in order to achieve (1) a safe and durable treatment, (2) FDA approval, and (3) health insurance coverage.

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A Timeline of NAAF’s Strategic Research Investments Toward a Treatment or a Cure for Alopecia Areata

- **1983**: NAAF’s Medical Advisory Board meets for the first time.
- **1985**: First research grant awarded.
- **1988**: First of 10 immunogenetic grants awarded to Dr. Madeleine Duvic.
- **1992**: First of 14 grants to develop an alopecia areata mouse model.
- **1997**: First of 12 immunogenetic grants awarded to Dr. Angela Christiano.
- **2000**: NIH funds creation of alopecia areata Registry, Biobank and Clinical Trials Networks. In 2012, NAAF assumes stewardship and becomes funder for the AA Registry, Biobank and Clinical Trials Network.
- **2010**: Dr. Christiano’s Genome Wide Association Study is published in *Nature*.
- **2012**: NAAF drives selection of alopecia areata as one of 39 diseases to be considered for targeted assistance through FDA’s Patient-Focused Drug Development Initiative.
- **2014**: Early trials at Columbia University of a drug based on rationally selected target informed by this new genetic and immunologic understanding show promise and efficacy for hair regrowth.
- **2015**: Dr. Christiano’s research findings, including the underlying genetic, immunologic, animal and human testing published in *Nature Medicine*.
- **2015**: FDA selects alopecia areata as one of few disease areas for a Patient-Focused Drug Development (PFDD) meeting during FY2016-2017 to enhance regulatory assessment and help innovative new drugs receive approval.
- **2016**: NAAF initiates Health and Research Ambassador program to educate and prepare the alopecia areata community to bring the patient voice into research design.
- **2016**: NAAF participates in first focus group with 6 bio-pharma companies are in early stage development of alopecia areata programs.
- **2016**: NAAF collaborates with industry to form a consortium with the intent of developing a single, consensus-defined patient-reported outcomes instrument for alopecia areata.
- **2017**: More than 370 participants engage in FDA’s Patient-Focused Drug Development (PFDD) meeting on alopecia areata to share their perspectives on treatments and how the disease impacts their lives.
- **2017**: NAAF participates in two FDA Critical Path Innovation Meetings on outcome assessments for alopecia areata.
- **2017**: NIH awards $5 million to establish an alopecia areata Center for Research Translation led by Dr. Angela Christiano at Columbia University.
- **2017**: FDA grants fast track designation for CTP-543, an oral JAK inhibitor for the treatment of alopecia areata; and orphan drug designation for HC017AA, a treatment for alopecia areata in pediatric patients.
- **2018**: First of 10 immunogenetic grants awarded to Dr. Madeleine Duvic.
- **2018**: First of 14 grants to develop an alopecia areata mouse model.
- **2018**: NAAF initiates Health and Research Ambassador program to educate and prepare the alopecia areata community to bring the patient voice into research design.
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The Market

A 2012 NAAF-coordinated alopecia areata incidence study revealed a cumulative lifetime incidence of 2.1 percent, which translates into 6.5 million people in the United States and 145 million worldwide who have, had, or will develop alopecia areata at some point in their lives. Alopecia areata can have a profound psychological impact on those affected by the disease and on their families. The sudden onset, recurrent episodes, and unpredictable course of alopecia areata can lead to difficulties at work, at school and in relationships. Depression, anxiety, and suicidal ideation are health issues that can accompany alopecia areata. The knowledge that current medical interventions are extremely limited and of minor effectiveness further exacerbates the emotional stresses experienced by patients.

NAAF has sponsored research summits that have established that alopecia areata is an autoimmune disease: it is in fact the most prevalent of these diseases, making it a priority candidate for research focus. Research has demonstrated genetic similarities with three other autoimmune diseases: type 1 diabetes, rheumatoid arthritis and celiac—all having significant health burdens and all involving end-organs that are not as easily accessed and studied as the skin and hair follicle. Alopecia areata as a disease represents a significant potential market for companies with effective treatments, and NAAF is uniquely positioned to facilitate and accelerate both basic and applied/clinical research to find safe, affordable treatments and a cure.

Even with these findings, and the fact that alopecia areata is the most prevalent autoimmune disease in the United States, research has lagged. This is in part because the disease is often viewed as appearance altering, but not life threatening. NAAF’s awareness and advocacy efforts to raise the profile of alopecia areata have helped to change that perception.

NAAF is widely regarded as the largest, most influential and most representative foundation associated with alopecia areata. NAAF is the premier organization coordinating research efforts, leveraging assets, acting as the trusted custodian for the Alopecia Areata Registry, Biobank and Clinical-Trials Network, and working on Capitol Hill to advocate for FDA approval and with CMS for insurance coverage. NAAF coordinates alopecia areata research worldwide.

Recent findings of potential treatments, along with NAAF’s work to raise awareness and pave the way for FDA approval, have contributed to a growing interest on the part of pharmaceutical companies to develop and bring to market therapies for alopecia areata. Through an Advocacy Day on Capitol Hill in 2012, as well as testimony at public meetings and individual meetings, NAAF drove the selection of alopecia areata as one of just 39 diseases (out of 12,000 diseases) to be considered for targeted assistance through the FDA’s Patient Focused Drug Development Initiative (PFDDI). In 2015, thanks to the tireless efforts of our Legislative Liaisons and our community at large, the FDA selected alopecia areata for a PFDDI meeting during FY2016—2017 to enhance regulatory assessment and help innovative new drugs receive approval. When the patient community informs regulatory decision making, it usually creates a more preferential risk-benefit assessment. A current congressional effort through the 21st Century Cures Initiative would recognize that the PFDDI has been a success and formally include the patient perspective every time FDA reviews an application for a new treatment moving forward.

NAAF works with pharmaceutical companies and our patient community to develop research that is focused on patient-centered clinical outcomes in order to ensure that the research into treatments is meaningful and useful to people living with alopecia areata. In 2012, NAAF coordinated and funded the development of a Core Uniform Protocol, the first step in a precedent-setting process by a patient advocacy group to facilitate and drive clinical research toward finding safe and effective treatments for alopecia areata. The protocol includes defined criteria for inclusions/exclusions, safety, and outcome assessment measures. Companies can plug in specific product data, enabling studies to move rapidly from concept to completion.

NAAF recently coordinated its first focus group with a pharmaceutical company interested in hearing the patient voice early on in their drug development process. We are currently in discussions with four additional pharmaceutical companies all of which are developing or expanding alopecia areata drug development programs. We will continue to work with our patient community, the scientific community, industry and the FDA to ensure the patient voice is considered during the research and development of new therapies for alopecia areata.
Treatment Development Program Action Plan

TDP is advancing the science through funding meritorious research, including (1) basic research on how alopecia areata triggers the body’s immune response and (2) translational and clinical studies to test the effectiveness of potential treatments. Grant applications undergo a two-step, single-blind review process under the guidance of a qualified scientific Chair. During initial merit review, teams of unconflicted scientists with relevant experience as well as patient stakeholders evaluate and score the applications for scientific and technical merit as well as relevance and importance to patients. An Integration Panel comprised of patient stakeholders, scientific experts, and NAAF staff then evaluates meritorious applications in light of funding priorities and budget constraints and makes funding recommendations to NAAF’s Board of Directors.

TDP also drives research by (1) hosting research summits bringing together experts in the fields of hair and skin disease research, clinical care, basic science, immunology, autoimmunity and industry to distill the science and chart a path for the most critical, high-leverage research investments; (2) building the infrastructure through the Alopecia Areata Registry, Biobank and Clinical Trials Network; (3) paving the way for FDA approval by developing a Uniform Clinical Trials Protocol, coordinating prevalence and incidence studies, and advocating on Capitol Hill; and (4) providing critical seed and bridge funding to nurture high-caliber, independent researchers with an enduring focus on research relevant to alopecia areata.

2015–2017 $2.9 Million TDP Budget Will Support:

- Alopecia Areata Registry: Grow the Alopecia Areata Registry from 4,000 samples to 10,000. With an expanded Registry, larger, more robust GWAS can be performed, which could potentially lead to even more precise targets and even safer treatment options for those with alopecia areata.

- Research Grant Program: Support clinical trials using treatments approved by the FDA for other indications, support promising research with a focus relevant to alopecia areata, and support highly scored and meritorious NIH research proposals worthy of bridge funding.

- The 2016 Research Summit: Bring together scientists, government representatives (FDA, CMS, NIH), and biopharmaceutical companies to facilitate partnerships and move promising research initiatives forward at record speed.

- Advocacy: Make sure that once an alopecia areata treatment is discovered and proven safe and durable, it will be approved by the FDA and reimbursed by Medicare so that all patients have access.

Discoveries achieved through the TDP will also contribute to a greater understanding of autoimmune diseases such as type 1 diabetes, rheumatoid arthritis and celiac disease. NAAF works to ensure that research findings are published and disseminated.

Program Evaluation

Each year, the NAAF Board of Directors meets to review progress toward TDP goals from the past year and establish goals and milestones for the next calendar year. Measurements of success include the following items: amount of leveraged funds from NIH, corporate engagement with biopharmaceutical companies, biennial Research Summit outcomes, NAAF-funded research progress and publications.

Over the next three years, NAAF expects to advance our basic immunology knowledge by exploring all avenues directed by worldwide immunology experts. We expect to grow the Registry with data and samples from 6,000 new participants. We also expect to have at least one treatment move from indication to approval and have more in line.
About National Alopecia Areata Foundation

Mission Statement: The National Alopecia Areata Foundation (NAAF) funds research to find a cure or acceptable treatment for alopecia areata, supports those with the disease, and educates the public about alopecia areata.

The mission is accomplished by:

- Funding and promoting research and research summits that add to the scientific knowledge about alopecia areata, its causes, and different treatments
- Providing support and education for people with alopecia areata and their families
- Informing the public about alopecia areata
- Advocating the concerns of people affected with alopecia areata
- Creating and distributing educational materials to health professionals as well as those affected, so that all may better understand alopecia areata.

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For information about how you can support the Treatment Development Program, please contact Maureen Smith, Chief Development Strategist, at maureen@naaf.org.