

The future of  
life sciences  
from challenge  
to change

GOLDEN  
BOOK





# A RECORD OF LEADERSHIP IN BRINGING THE BEST OF BIOMEDICAL DISCOVERY TO THE ATTENTION OF THE WORLD...



“The Prix Galien is a welcome initiative to stimulate creative research and promote excellence.”

**Barack Obama** - 2008



“I know the importance of the Prix Galien which rewards every year therapeutic innovations and research works among the most creative.”

**Emmanuel Macron** - 2018



“The Prix Galien since 1970 is dedicated to promoting the research and development of the medicine and medicine industries.”

**Macky Sall** - 2018



“The Prix Galien is the right event, on the right issue at the right time. I thank the Galien Foundation for bringing us together and for recognizing that a healthier world is a safer world and a more just world.”

**BAN Ki-moon** - 2010



“It is a pleasure to congratulate all those who promote the Prix Galien and pharmaceutical research: they play a vital role in the fight against disease throughout the world.”

**John Major** - 1996



“I welcome you to the annual Prix Galien awards for innovative excellence in biomedical products and health technologies. The awards are among the highest honors in science and commerce.”

**Jimmy Carter** - 2017



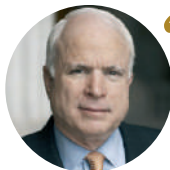
“I am particularly grateful to receive this award and I will treasure it.”

**Bill Clinton** - 2010



“The Prix Galien Awards are a true celebration of the years of hard work that are required to produce life-changing interventions.”

**Bill & Melinda Gates** - 2018



“I applaud Prix Galien for recognizing the pioneering spirit of America innovation and research.”

**John McCain\***

American Senator - 2008

*\*In Memoriam*



“I look forward to the day when we will celebrate an African winner of the Prix Galien.”

**Tedros Adhanom Ghebreyesus**

2018



**THE PRIX GALIEN AWARD RECOGNIZES OUTSTANDING ACHIEVEMENTS IN IMPROVING THE GLOBAL HUMAN CONDITION THROUGH THE DEVELOPMENT OF INNOVATIVE DRUGS AND OTHER TREATMENTS.**

**THE PRIX GALIEN WAS CREATED IN 1970 IN HONOR OF GALEN, THE FATHER OF MEDICAL SCIENCE AND MODERN PHARMACOLOGY.**

**WORLDWIDE THE PRIX GALIEN IS REGARDED AS THE EQUIVALENT OF THE NOBEL PRIZE IN BIOPHARMACEUTICAL RESEARCH.**



*Sue Desmond-Hellmann, Ethiopia Clinic site visit  
 Former CEO, The Bill & Melinda Gates Foundation  
 Committee Chair of Prix Galien USA  
 and Prix Galien International*

**OUR MISSION:**

The Prix Galien is more than an award: it is a movement with a mandate to foster, recognize and reward excellence in scientific innovation to improve the state of human health. Building on an unrivaled network of top biomedical scientists including Nobel Laureates in medicine, the Prix Galien manages an independent, cross-functional and geographically diverse program of events and sponsorships to brand “the best of the best” in new medicines and diagnostics.

Our scope is global, and our commitment to progress in medicine is both measurable and concrete. Our members express this through the establishment of productive relationships to build lasting bridges between the commercial research enterprise and local communities engaged in public policy, science, finance, academic research and the media.

In addition to recognizing advances in promising therapies, the Roy Vagelos Pro Bono Humanum Award for global equity brings a unique focus to the intersection between science, business and politics.

The outcome we seek is guided by the synthesis principle that underpins the conduct of science itself: successful innovation where financing, physical assets, knowledge and skills are combined from many sources to move new ideas quickly “from the bench to the bedside,” on behalf of patients everywhere.

**A truly global program present in 14 countries\***

Our program includes a review of contributions from a new generation of innovators representing diverse sectors in health. Our theme: better cross-cultural contacts, harmonized regulation, internal business process improvements, new information technologies and effective public-private partnerships can remove barriers to the commercialization of good medicines and expand access to these benefits to all who need them.

**2022**

Through our initiative in Africa and the celebration of our Golden Jubilee in Dakar, Senegal, 2021 will represent a new and exciting stage in the evolution of the Prix Galien as the pre-eminent “force populaire” behind the global scientific enterprise. With the support of our sponsoring organizations, our efforts will extend to all who see medical innovation not only as an industrial policy asset but as a source of social progress – where private enterprise and public engagement combine to deliver a greater public good.

We invite all communities with a commitment to the conduct and promotion of life sciences innovation to contribute to this important work.

\* Belgium, Canada, China, France, Germany, Greece, Italy, Netherlands, Poland, Russia, Spain, Switzerland, United Kingdom and the United States



Elie WIESEL In Memoriam  
Honorary Founding President  
The Galien Foundation  
Peace Nobel Laureate  
Boston University

PRIX GALIEN USA

FIRST COMMITTEE 2007-2011



Michael S. BROWN, MD  
Nobel Laureate  
Professor of Molecular  
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UT Southwestern  
Medical Center at Dallas



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Vice President for  
Medical Affairs  
and Physician-in-Chief,  
Rockefeller University



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Professor Emeritus  
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Scripps Research Institute



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Cancer Center



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Professor Karolinska Institutet;  
Former President Karolinska  
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Nobel Foundation



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New York University School  
of Medicine



Gerald WEISSMANN, M.D.  
Professor of Rheumatology  
and Director of Biotechnology  
Study Center  
New York University School  
of Medicine

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2022



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Committee Chair  
Former Chief Executive Officer  
Bill & Melinda Gates Foundation



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Medical Center



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Fred Hutchinson Cancer  
Research Center member,  
and Professor of Physiology  
and Biophysics  
University of Washington



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President and CEO Dana Farber  
Cancer Institute;  
Professor of Medicine  
Harvard Medical School



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*Peace Nobel Laureate,*  
Boston University,  
*Honorary Founding  
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University of Washington



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David H. Koch Institute  
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Massachusetts Institute  
of Technology



**Cato T. LAURENCIN, MD, PhD**  
University Professor  
University of Connecticut



**P. Roy VAGELOS**  
*Committee Chair 2012-2017*  
*Retired Chairman and CEO,*  
*Merck & Co., Inc. Chairman of the Board,*  
*Regeneron Pharmaceuticals, Inc.*

**ROY VAGELOS**  
PRO BONO HUMANUM  
**AWARD**  
FOR GLOBAL HEALTH EQUITY



**Steven LESTER, MD**  
Chief Medical Officer  
Mayo Clinic-ASU  
MedTech Accelerator



**Michael ROSENBLATT, MD**  
Senior Partner  
Flagship Pioneering



**Marc TESSIER-LAVIGNE, PhD**  
President  
Stanford University

ROY VAGELOS  
PRO BONO HUMANUM  
AWARD



FOR GLOBAL HEALTH EQUITY





Elie Wiesel, Peace Nobel Laureate; BAN Ki-moon, General Secretary, United Nations; Bill Clinton & Philippe Douste-Blazy, 2010 Prix Galien Pro Bono Humanum Award Laureate

## AN UNRIVALED REPUTATION FOR INTELLECTUAL RIGOR AND INTEGRITY

THE COMMITTEE FOLLOWS A CENTURY-OLD EXAMPLE SET IN SWEDEN. THE PRIX GALIEN REWARDS EXCELLENCE AND NOVELTY, AND AWARDS SPECIAL HONORS TO THOSE WHO HAVE SERVED HUMANITY AT LARGE. PRESENTED AT THE CEREMONY, THE PRO BONO HUMANUM AWARD RECOGNIZES EXEMPLARY AND INNOVATIVE EFFORTS IN IMPROVING THE HUMAN CONDITION. THIS SPECIAL AWARD WAS PRESENTED BY THE LATE AND BELOVED ELIE WIESEL, IN PRESENCE OF THE PRIX GALIEN USA COMMITTEE. THE PRO BONO HUMANUM AWARD WAS RENAMED THE ROY VAGELOS PRO BONO HUMANUM AWARD IN 2017 IN RECOGNITION OF HIS DECADES OF LEADERSHIP IN IMPROVING THE STATE OF HUMAN HEALTH THROUGH MEDICINES INNOVATION.

### **P. Roy Vagelos**

Prix Galien USA Committee Chair 2012-2017

Retired Chairman and CEO, Merck & Co., Inc.  
Chairman of the Board, Regeneron Pharmaceuticals, Inc.





2022  
ROY VAGELOS  
PRO BONO HUMANUM  
AWARD  
FOR GLOBAL HEALTH EQUITY



To  
STANLEY T. CROOKE

**In recognition of his nearly 50 years of dedication** to a novel, clinically endorsed concept of using synthetic oligonucleotides to control the expression of genes and proteins that impact disease. His singular dedication to this RNA-driven antisense platform provided a new, highly personalized pathway to resolving some of the most difficult biomedical challenges, with the ultimate potential of treating multiple infections, inflammatory conditions and cancers targeted to individual patient profiles and with minimal side-effects. Due to Dr. Crooke's years of effort as a researcher, corporate R&D executive, biotech innovator and philanthropist, nearly a dozen antisense oligonucleotides (ASO) have been

approved for clinical use since 1998; additional clinical trials focused in high unmet medical need areas like neuroscience are underway.

Dr. Crooke is also recognized for his leadership in being among the first in industry to make a commitment to open science a key element of the modern R&D enterprise and to embrace the principle that even patients with the rarest of afflictions deserve the best talent and expertise our R&D industry can offer. Untold thousands of patients formerly with no hope of a cure stand to benefit from his belief in the promise of this entirely new class of medicines.



PRO BONO HUMANUM  
**LAUREATES**  
SINCE 2007

**2021**

In recognition of the monumental feat of having developed, in less than a year, vaccines against COVID-19, as well as having developed several novel antiviral drugs that treat COVID-19 symptoms based on innovative monoclonal antibody platforms. The six companies together responded to the pandemic in record time, with safe, effective treatments that hold the promise of reducing mortality and restoring normal life for untold millions of vulnerable patients.



**Stephane Bancel**  
CEO, Moderna



**Albert Bourla**  
Chairman & CEO,  
Pfizer



**Alex Gorsky**  
Chairman, Board  
of Directors &  
CEO, Johnson &  
Johnson



**David Ricks**  
Chairman & CEO,  
Eli Lilly & Co.



**Pascal Soriot**  
CEO,  
Astra Zeneca



**George D.  
Yancopoulos**  
Co-Founder, President  
& Chief Scientific  
Officer, Regeneron



**2020**

**Dr. William H. Foege**

In recognition of his role in building the global strategy that resulted in the eradication of this smallpox virus in 1980, a success that helped foster higher rates of immunization against many

other infectious and communicable diseases throughout the developing world.



© Tony Lopez

**2019**

**Jim and Marilyn Simons**  
The Simons Foundation

In recognition of their proactive support of basic scientific research undertaken in the pursuit of understanding the phenomena

of our world, often including research by scientists in the early stages of their careers, a new collaborative funding model to facilitate cross-disciplinary breakthroughs, and efforts to inspire emerging and current philanthropists to dedicate a portion of their philanthropy to basic science.



**2018**

**Bill and Melinda Gates**  
Bill & Melinda Gates Foundation

In recognition of their extraordinary work championing scientific and technological innovation to

improve life for the world's poorest people. As co-chairs of the Bill & Melinda Gates Foundation, they have catalyzed unprecedented progress in global health, poverty reduction, and public education in the U.S.



**2017**

**Jimmy Carter**  
Former U.S. President &  
Co-Founder, The Carter Center

For playing a leadership role in the elimination of river blindness in four of the six

Latin American countries where it was endemic and halting the disease's transmission in several locations in Africa where more than 99 percent of the global cases exist.



**2016**

**Paula S. Apsell**  
Executive Producer, PBS NOVA  
series & Director, Science Unit,  
WGBH

For turning great science into a human story, showing

that scientific literacy is society's first line of defense against the destructive forces of fear and ignorance that challenge further progress in human health.



## 2015

### Pr. Mary-Claire King

Professor, Genome Sciences and Medical Genetics, University of Washington

In recognition of her work in transforming the application of human genetics to medicine through identification of the first gene, BRCA1, responsible for inherited susceptibility to breast cancer. And for pioneering the application of genetic sequencing in forensics to identify victims of human rights abuse.

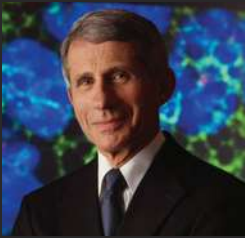


## 2014

### Dr. Bernard Kouchner

Co-Founder, Doctors Without Border & Former French Minister of Foreign and European Affairs

In recognition of his role in the creation, from amongst French doctors, this group of "Doctors Without Borders" who have lent the modern field of humanitarian medicine both legitimacy and worldwide acclaim and for his fieldwork that has nurtured and developed, not only among public opinion but also within various governments, the notion of the universality of humanitarian action and the concept of the Non-Governmental Organization.



## 2013

### Dr. Anthony Fauci

Director, U.S. National Institute of Allergy and Infectious Diseases

For his paramount contributions to basic and clinical research on the pathogenesis and treatment of immune mediated and infectious diseases.



## 2012

### Dr. Francis S. Collins

Director, U.S. National Institutes of Health

Dr. Collins, Director of the National Institutes of Health (N.I.H). In recognition of his contribution to the ethical implications of scientific research.



## 2011

### Pr. Paul Farmer

Chief Strategist and Co-Founder, Partners In Health

For his humanitarian work in Haiti and throughout the world, including advancing the fight against infectious diseases, including AIDS and tuberculosis.



## 2010

### Bill Clinton and Philippe Douste-Blazy

Founder, Clinton Foundation and Former U.S. President; Chairman, UNITAID

In recognition of their achievements in providing treatment and increasing access to medicines for underserved populations through the UNITAID and Clinton Foundation HIV/AIDS Initiative (CHAI) partnership.



## 2009

### Barry Bloom and Jeffrey Sachs

Professor of Public Health, Harvard University; Director, Earth Institute, Columbia University

Harvard Professor Barry Bloom for bringing the fruits of basic biological science to those who are most needy. Columbia University Earth Institute Director Jeffrey Sachs for bringing the fruits of scholarly economics to bear our problems that have plagued the world for millennia.



## 2008

### Sheldon Segal and the Population Council

Former Chairman, Population Council

For their global efforts in support of reproductive health and family planning.



## 2007

### P. Roy Vagelos

Retired Chairman and CEO Merck & Co., Inc.; Chairman of the Board Regeneron Pharmaceuticals, Inc.

For the River Blindness Program and his historic decision to donate the drug Mectizan to more than 530 million people in 34 countries to treat and prevent river blindness "as much as necessary for as long as necessary."

Stan,  
our heartfelt congrats on  
receiving the 2022 Roy  
Vagelos Pro Bono Humanum  
Award for Global Health  
Equity.

This prestigious prize is a  
well merited recognition of  
your exemplary dedication  
to improving the lives of  
sufferers from  
rare diseases worldwide.



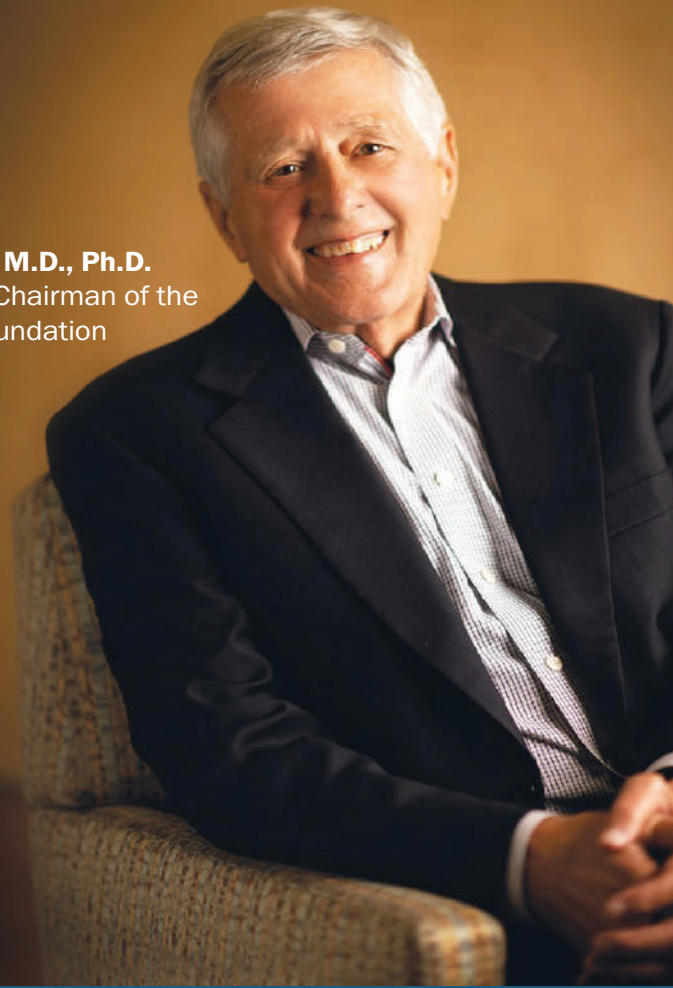
**Stanley T. Crooke, MD, PhD.**  
*Founder, Chief Executive Officer and Chairman of the Board*  
**n-Lorem Foundation**

**IONIS**



where  
**science** meets **humanity**™

**Stanley T. Crooke, M.D., Ph.D.**  
Founder, CEO and Chairman of the  
Board, n-Lorem Foundation



*Bravo,  
Stan!*

Congratulations on receiving the Roy Vagelos, Pro Bono Humanum Award for Global Health Equity. Your vision, passion and dedication has brought solutions to thousands of patients and has shaped our industry.

**I am proud of our longtime collaboration.**

*Michel Vounatsos*

CEO of Biogen

# Congratulations Stan!!!

on receiving the 2022 Roy Vagelos Pro Bono  
Humanum Award for Global Health Equity




*John Maraganore*

Former Founding CEO  
Alynlam Pharmaceuticals



**Stanley T. Crooke, MD, PhD.**

Founder, Chief Executive Officer and Chairman of the Board  
n-Lorem Foundation

A portrait of Dr. Stanley T. Crooke, an older man with grey hair, wearing a dark suit jacket over a light-colored shirt. He is sitting in a chair and smiling. The background is a warm, golden-brown color.

**Those with the Vision  
and Courage to Leap  
Forward will Lead the  
Way to Transform the  
Human Condition.**

**Congratulations to Dr. Stanley T. Crooke from IONIS on receiving the Roy Vagelos Pro Bono Humanum Award for Global Health Equity.**

For almost 50 years, your determination, dedication, and persistence have fueled your vision for advancing antisense technology to discover, develop, and deliver a broad range of novel medicines to address unmet medical needs. These groundbreaking medicines include SPINRAZA® (nusinersen), the first approved, disease-modifying therapy for the treatment of spinal muscular atrophy (SMA) which has been nominated for Best Rare/Orphan Drug at tonight's Prix Galien Awards.

*Your passion, scientific rigor, commitment to open science, and constant focus on the patient have been a driving force in the biopharmaceutical industry. Your leadership has driven IONIS to foster a unique culture dedicated to continuously discovering and advancing novel, RNA-targeted therapies with the potential to transform lives.*

We are honored to have been a part of creating an entirely new class of medicines.

**IONIS**<sup>®</sup>

A graphic element in the bottom right corner consisting of several overlapping, curved, diagonal stripes in shades of purple, magenta, and orange.





**SPINRAZA® has brought hope to spinal muscular atrophy (SMA) patients and their families, transforming the lives of thousands worldwide.**

**We could not have accomplished this without the SMA Community, including the caregivers and healthcare providers who tirelessly support patients and their families. At IONIS we know that sick people depend on us. That is why we are committed to applying our skills and scientific expertise to discover, develop, and deliver novel therapies with the potential to transform patients' lives.**

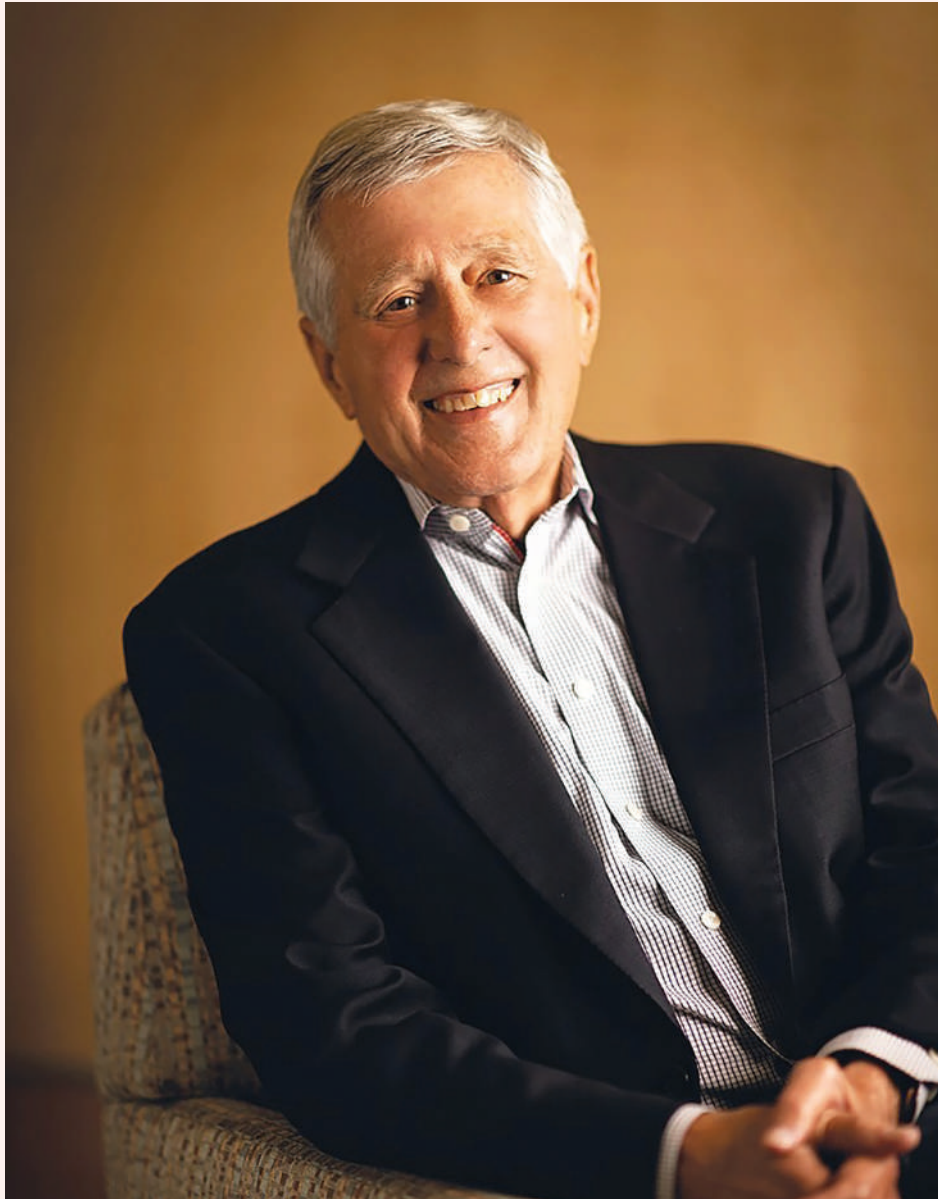


IONIS is honored that SPINRAZA, the global foundation of care for the treatment of SMA, has been nominated for "Best Rare/Orphan Drug" and joins in celebrating the Galien Foundation recognizing those innovative products that have improved the human condition over the last 50 years. We give special thanks to Dr. Stanley T. Crooke, Dr. Frank Bennett, IONIS, Dr. Adrian Krainer, Cold Spring Harbor Laboratory, and Biogen for their participation in the discovery and development of SPINRAZA (nusinersen), the first disease-modifying therapy to treat SMA.

**We congratulate all the nominees and winners.**

**IONIS®**





# Stanley T. Crooke M.D., Ph.D.

Founder, Chairman of the Board and CEO,  
**n-Lorem Foundation**

## Dear Colleagues —

On behalf of the patients we serve at n-Lorem, I am humbled and honored to accept the Roy Vagelos Pro Bono Humanum award. Though n-Lorem is only three years old, the effort that makes n-Lorem possible has required more than three decades of integrated innovation that I had the privilege to lead in the service of patients. That commitment to innovation continues to advance the technology and is a core value at n-Lorem. The technology that enables n-Lorem is RNA-targeted drug discovery, a concept that was considered impossible for many of the years in which we developed it. The efficiency, versatility, rapidity and cost effectiveness of antisense technology supports a new chapter that was literally inconceivable before the creation and advancement of the technology. At n-Lorem, we are establishing a new drug discovery and development program designed to meet the needs of single patients and providing experimental ASO treatments to patients for free for life. As impossible as that may sound, we have proven that it is possible.

Though we have made great progress, we know that we have a long journey ahead with many challenges. But, we are buoyed by the support from individual donors, foundations, a growing number of biotechnology and pharmaceutical companies and many vendors to who our industry who have joined in this journey of discovery for mind and the heart. We know we are privileged to make this journey.

I am privileged to accept this award. I do so on behalf of our patients, my colleagues at n-Lorem and all the supporters who are making this marvelous journey possible. We thank the Prix Galien Awards Committee for this honor and commit to continue to earn the recognition as we work to meet the needs of many nano-rare patients.

With gratitude,



**Stanley T. Crooke, M.D., Ph.D.**

Founder, Chairman of the Board and CEO, n-Lorem Foundation

Founder, Former Chairman of the Board and CEO of Ionis Pharmaceuticals

# Quality Established





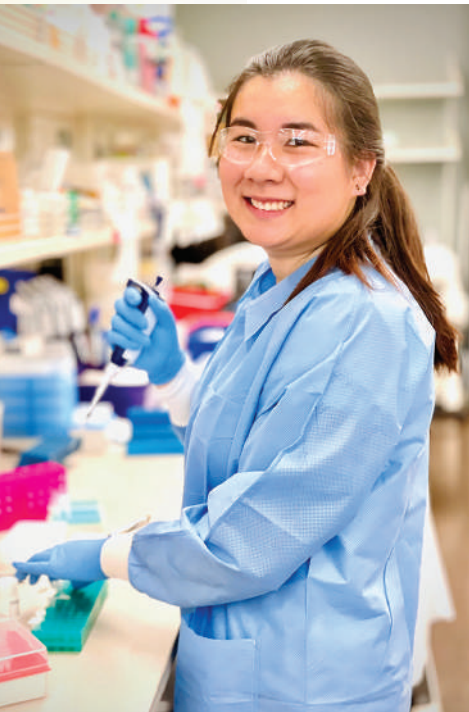
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### A Commitment to Desperate Patients

Our mission is to provide personalized experimental ASO medicines to treat nano-rare patients —**for free, for life.**

ASO technology is the **ONLY** technology with regulatory guidance to treat nano-rare patients.

We have worked closely with the FDA for approaches to the treatment of nano-rare disease patients. We are deeply appreciative of the guidance that addresses the unique challenges these patients present and ambitiously pursue the treatments they deserve.





Hopelessness  
to hope

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We work with those who  
need the most help—  
the nano-rare community

“I felt like I was fighting for so many years for my son, especially in the beginning, and now I’m watching these amazing people show up and work on this personalized treatment for one child that could potentially benefit and change the course of his life and our family’s life. Just that is enough – it’s the best feeling, it’s hope.”

— KELLY DALBY / MOTHER OF CONNOR

# Helplessness to help

# We need your help changing the world

We provide an opportunity for a better life, renewed hope and brighter dreams, one patient, one family at a time. We believe that is a great way to change the world.

To learn more and donate, please visit:  
[www.nlorem.org](http://www.nlorem.org)

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## n-Lorem Today

**70+**

More than 70 nano-rare patients accepted for treatment

**30+**

More than 30 years of investment and innovation in ASO technology

**4-8**

4-8 nano-rare patients to be treated in 2022





A 'Corps of  
Discovery' for the  
mind and heart



