

## Who We Are

**Ionis unites groundbreaking science and technology with relentless passion to discover and deliver medicines that enable better futures for people living with serious diseases.**

For more than three decades, Ionis has pioneered RNA therapeutics to achieve firsts that were once thought impossible. We have discovered multiple marketed medicines and have a leading pipeline in neurology, cardiology and other areas of high patient need. Our pipeline is fueled by relentless innovation of our science and technology, which has enhanced the profiles of our existing RNA-targeted medicines and unlocked new opportunities in emerging areas such as gene editing. Our commitment to changing lives is a calling that pushes us to deliver the next solution for patients and those who care for them.

**Locations:**  
Carlsbad, CA  
(headquarters)  
Boston, MA  
Dublin, Ireland

**Founded:**  
1989

**Employees:**  
1,110+

## Our Science

Ionis' science is driven by a deep understanding of the genetic foundations of diseases and a commitment to advancing next-generation technology, coupled with an urgency to deliver medical breakthroughs for patients in need.

Ionis is grounded in a heritage of RNA-targeted medicines. We forged the way in the now-proven field of oligonucleotide therapeutics with award-winning and groundbreaking approaches to treating serious diseases.

- We turned spinal muscular atrophy, a rare, progressive and deadly genetic disease, into a chronic condition. Infants who would not have reached their second birthday now have the potential to reach adulthood.
- Our science and persistence also led to the first medicine for a genetic cause of amyotrophic lateral sclerosis.

We developed methods to control gene expression, including silencing harmful genes and boosting the activity of genes to compensate for missing or damaged ones.

We continue to advance and expand our chemistry expertise, enabling us to accurately identify the appropriate medicines for specific diseases, maximizing their impact and effectiveness and ultimately, delivering transformative medicines to patients.

## Approved Medicines

Ionis discovered and developed **multiple marketed medicines** for serious diseases, including breakthrough medicines for cardiovascular and neurologic diseases.



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## Our Expansive Late-Stage Pipeline

We've built an impressive pipeline and have set our sights on delivering important new medicines for people with rare and challenging diseases who are in critical need of significant advances, particularly in our focus areas of neurology and cardiology.

Our science and technology have led to a rich pipeline of medicines for approximately **20 serious diseases that have the potential to benefit people living with these devastating conditions.**

Our late-stage pipeline has **several medicines in Phase 3 development for 8 diseases** and including **independent launches.**

## Corporate Responsibility

Ionis' approach to corporate responsibility supports our goal of delivering next-level value for patients and all stakeholders. We aim to:

**Innovate to improve the lives of people with serious diseases** – We innovate across the business and work tirelessly to discover, develop and deliver important new medicines for people with serious diseases.

**Empower our people and communities** – We are committed to fostering an inclusive culture that drives excellence, embraces diversity and supports our communities.

**Operate responsibly and sustainably** – We operate with integrity to help create a better, more sustainable future for all through environmental stewardship, responsible business practices and stakeholder interactions.

For more, please see our latest [Corporate Responsibility Report](#).

## Better Futures, Secured

We are building on our rich history, and believe our greatest achievements are ahead of us. We are continuing to progress the development and delivery important new medicines, including our first independent launches, specifically targeting rare and challenging diseases for people who are in critical need of significant advancements.

For more information about Ionis, please visit [ionis.com](https://ionis.com) and engage with us on [X \(Twitter\)](#) [@ionispharma](#), [Instagram @ionis\\_pharma](#) and on [LinkedIn](#).