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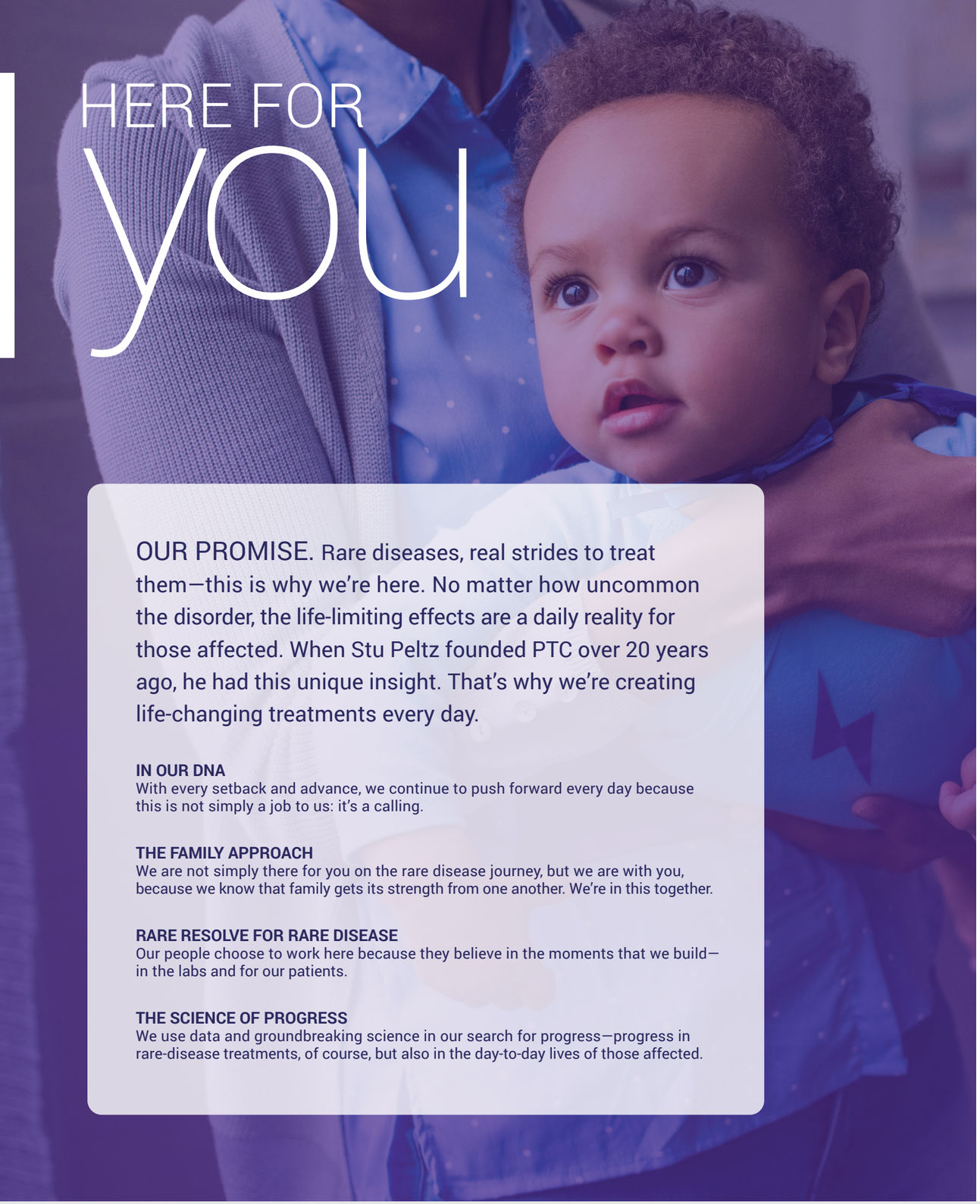
2020 Annual Report



HERE FOR *you*

OUR PROMISE. Rare diseases, real strides to treat them—this is why we're here. No matter how uncommon the disorder, the life-limiting effects are a daily reality for those affected. When Stu Peltz founded PTC over 20 years ago, he had this unique insight. That's why we're creating life-changing treatments every day.

IN OUR DNA
With every setback and advance, we continue to push forward every day because this is not simply a job to us: it's a calling.

THE FAMILY APPROACH
We are not simply there for you on the rare disease journey, but we are with you, because we know that family gets its strength from one another. We're in this together.

RARE RESOLVE FOR RARE DISEASE
Our people choose to work here because they believe in the moments that we build—in the labs and for our patients.

THE SCIENCE OF PROGRESS
We use data and groundbreaking science in our search for progress—progress in rare-disease treatments, of course, but also in the day-to-day lives of those affected.

A MESSAGE TO OUR SHAREHOLDERS

Stuart W. Peltz, Ph.D., *Chief Executive Officer*

2020 was a year like no other in our lifetime. It was marked by a global pandemic that impacted all of us and made life more challenging on both personal and professional levels. Even in the face of these issues, however, I am pleased to report that PTC rose to the occasion, making 2020 an incredibly productive and successful year. Having just celebrated our 23rd anniversary, I am proud that our company is better positioned to drive innovation and capitalize on our diversified and robust scientific platforms and programs.

Even at the beginning of the pandemic, PTC was able to pivot quickly and adjust the way we worked in the "new normal" environment. We recognized the potential issues that a pandemic would cause and closed our facilities earlier than almost any other company, stopping all non-essential business travel in late February and subsequently went to fully remote operations within the first week of March last year. We created several task forces to deal with multiple aspects of the crisis, with the emphasis on continuing to be productive and to ensure patients would still receive their therapies. New approaches to communicate with employees were developed, with weekly video messages and constant and consistent transparent public discussions. The safety of our employees and our patients was paramount in all the decisions we made.

Clinical trials became a particular challenge, but we had an advantage because most of our therapies in development are small molecule drugs that are orally administered. This means no injection or infusion is needed, so that dangerous trips to hospitals overwhelmed with the COVID-19 response were not necessary. Therefore overall, we were uniquely positioned to ensure the continuation of our clinical trials even in these most difficult times. We were able to initiate five clinical trials, including three registration-directed trials, of which two are from our Bio-e platform.

We have had substantial revenue growth this year and have increased our capabilities to discover, develop and commercialize our diversified pipeline. As the pandemic unfolded, we suspended the financial guidance for the year. Nonetheless, I am proud to report that revenues for the year were in line with our pre-pandemic estimates, with 14 percent year-over-year growth, and total net product revenue was $331 million. The increased revenue was driven by our Duchenne muscular dystrophy franchise consisting of Translarna™ (ataluren) and Emflaza® (deflazacort). Of note, revenue for Emflaza increased 38 percent year-over-year. Translarna's growth was driven by new patients in existing geographies, geographic expansion, and label updates driving broader access. The revenue generated helps drive the continued investment in developing new treatments for patients with high unmet need.

I am incredibly proud of how we weathered the storm. It is a testament to our employees -- their ingenuity and

"Having just celebrated our 23rd anniversary, I am proud that our company is better positioned to drive innovation and capitalize on our diversified and robust scientific platforms and programs."

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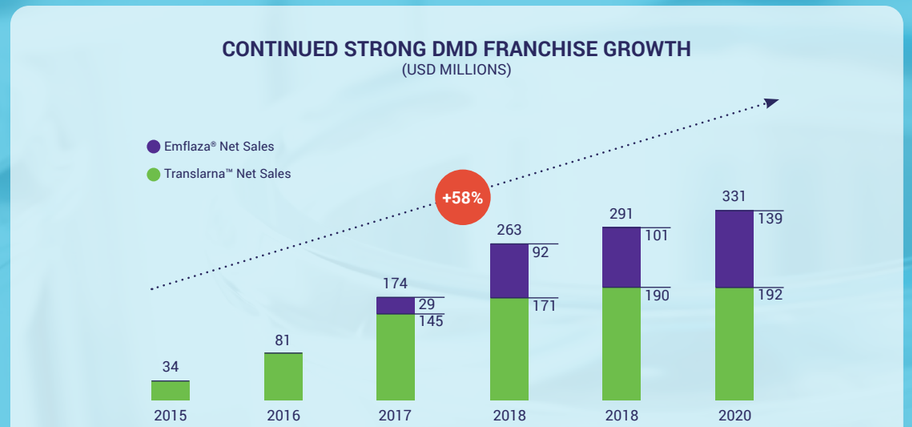
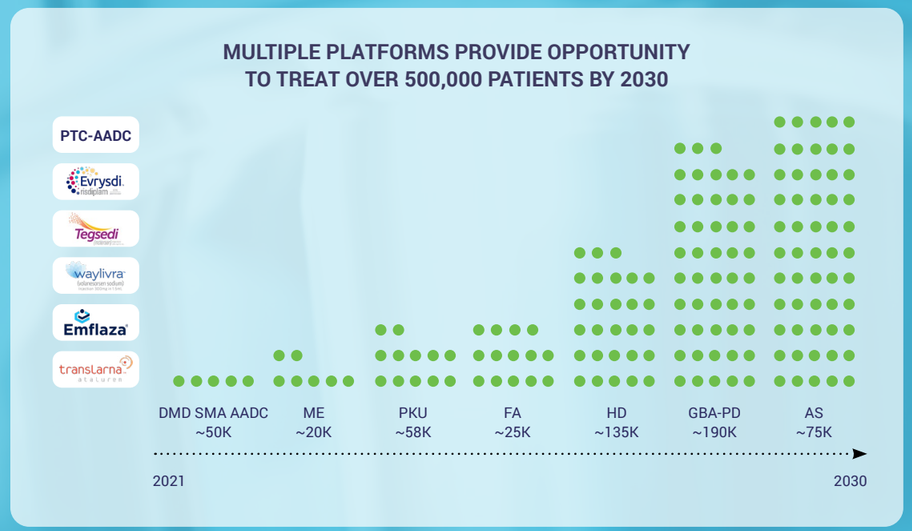
DRIVING results





perseverance in the face of multiple challenges; their resilience and adaptability as circumstances changed; and their passion for our mission to provide innovative treatments to patients with debilitating rare diseases that have few or no treatment options. PTC has emerged a stronger and more resilient company.

Perhaps the most significant milestone achieved in 2020 was in August with the FDA approval of Evrysdi™ (risdiplam), the first at-home, orally administered treatment for spinal muscular atrophy (SMA) in adults and children two months and older. SMA is a devastating neuromuscular disease that is the leading genetic cause of death in infants and young children. Evrysdi was discovered from PTC's splicing platform and was developed in collaboration with Roche and the SMA Foundation.

The development of Evrysdi provides an example of the level of dedication and perseverance that is a hallmark of PTC. Back in 2005, the SMA Foundation approached us to develop therapies for SMA. One program was the identification of molecules that selectively and specifically affect splicing. We screened our compound library of more than 300,000 molecules. The program had well over fifty scientists including biologists, chemists, pharmacologists, and toxicologists. It also took enormous resources and dedication from our collaboration partners, including the SMA Foundation and in 2011 F. Hoffmann La Roche Ltd. (Roche), who joined us in this effort.

More recently, the European Commission approved Evrysdi for SMA patients two months and older. Roche has stated publicly that Evrysdi is expected to become the treatment of choice for SMA patients in the United States in 2021, and we are pleased to have brought such a game-changing therapy to market. While our journey was not always straightforward, we believe that the results of bringing such an important therapy to patients with such high unmet need are a testament to all that makes PTC great.

We continue to build on our splicing platform and have multiple programs based on this technology. I am happy to report that another development candidate from our splicing platform, PTC518, has moved into clinical development in 2020. PTC518 is being developed as a potential treatment for Huntington's disease. PTC518 alters huntingtin (HTT) splicing that causes the reduction of HTT levels, which is critically important for treating Huntington's disease. We recently shared our preliminary results from the phase 1 healthy volunteer trial. We are pleased to report that PTC518 treatment resulted in dose-dependent reduction of HTT messenger RNA levels. PTC518 was well-tolerated at all dose levels with no safety findings observed. Like risdiplam, it is a small molecule that distributes to all tissues in the body and therefore PTC518 is able to get to all tissues in the brain. This is a critical attribute of PTC518 because of the neurodegenerative nature of Huntington's disease. We feel confident that, with our experience with SMA drug development, we are well positioned for success to drive PTC518 development to treat Huntington's Disease.

Let me turn to our gene therapy platform to treat rare monogenic diseases. Our most advanced gene therapy product, PTC-AADC, is a gene therapy for the treatment of Aromatic L-Amino Acid Decarboxylase, or AADC deficiency. In 2020, we submitted a marketing authorization application to European regulatory authorities and expect an opinion from the EMA's Committee for

"I am incredibly proud of how we weathered the storm. It is a testament to our employees -- their ingenuity and perseverance in the face of multiple challenges."

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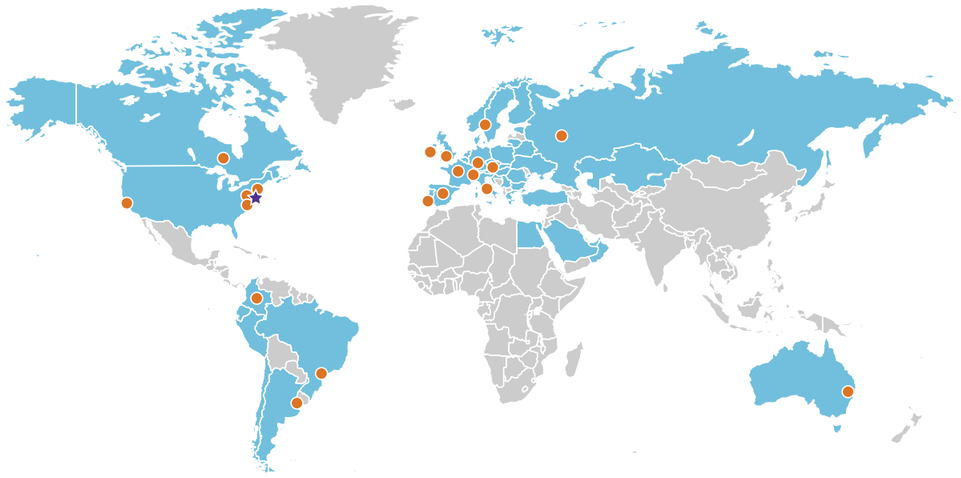
Global Geographic Presence Supports Growing Product Portfolio

OFFICES IN
20
COUNTRIES

FOOTPRINT IN MORE THAN
50
COUNTRIES

1K
EMPLOYEES



Significant Execution & Value Creation In 2020

CLINICAL

- Initiated two potential registrational trials with vatiquinone in Mitochondrial Epilepsy & Friedreich ataxia.
- Completed Translarna™ dystrophin trial for potential US accelerated approval
- Initiated trial of PTC518 in healthy volunteers for Huntington's disease program

REGULATORY

- Evrysdi™ approval in US and multiple additional countries
- Submitted MAA to EMA for gene therapy to treat AADC deficiency
- Translarna™ label modification related to non-ambulatory patients

COMMERCIAL

- Broader patient access and continued geographic growth of Translarna™
- Strong Emflaza® growth: 38% year-over-year
- Evrysdi™ strong commercial launch

FINANCIAL

- Strengthened balance sheet; over **$1B** cash position
- **$333M Net Product Revenue**
- **$331 DMD Franchise Net Product Revenue**
- **$42.5M Roche Collaboration Revenue** associated with Evrysdi™ regulatory and sales milestones

Medicinal Products for Human Use (CHMP). We are also preparing a biologics license application for PTC-AADC for the treatment of AADC deficiency in the United States.

We had been concerned with the issues that have arisen because of the pandemic. We recognized the devastation of the lost jobs and economic havoc that the pandemic was causing, which included the impact of the constricted job market on recent university graduates. To address this problem, we launched an intern program for recent graduates. We hired 53 interns with diverse backgrounds, and they are currently working across all aspects of the company. They are doing important work and have shown enthusiasm, commitment, and passion. It has been inspiring to watch them grow. We are proud to help support the next generation of intelligent and highly motivated individuals who can become the next generation leaders in the biotech industry.

We also looked within our pipeline to determine whether we have potential therapies to treat COVID-19 patients. PTC299, a dihydroorotate dehydrogenase (DHODH) inhibitor, was shown to be a potential treatment. PTC299 targets two key elements of the virus infection, viral replication and the uncontrolled inflammatory response. Another benefit is that DHODH is a cellular enzyme, making it less likely to elicit drug resistance and not be limited by the variants. While great strides have been made in vaccine development, the lack of effective COVID-19 treatments has significantly hampered our ability to resume normal life. In 2020, we were able to rapidly initiate a Phase 2/3 registrational trial of PTC299 for COVID-19.

The progress made in 2020 is a testament to PTC's culture and its people. We strive to create a culture based on trust, respect, and inclusion. Our employees behaved as "one PTC"—a team that is passionate about purpose and focused on bold action. Their commitment pushes us to always do better, and to be better. In fact, at a time where employees could have disengaged with their jobs and the company, we saw record engagement that surpassed standards observed across our industry. It is a testament to our commitment and a desire to want to do great things for all our stakeholders.

We now have employees in over 20 countries around the globe, making diversity a natural element of our culture. We continue to foster our diverse and talented group of professionals and develop them so that they can continue to grow and tackle new responsibilities. In 2020, we formalized our Equality, Diversity and Inclusion (ED&I) initiatives and hired a dynamic leader who will bring our efforts even further, bringing new opportunities to women, minorities, and other underrepresented groups early and often, through education, mentorship, and career flexibility which is core to our focus.

This, too, is part of what makes PTC the company it is, and why I believe, building on our success in 2020, we are poised for an even brighter future.

Sincerely,

Stuart W. Peltz, PhD
Chief Executive Officer

"The progress made in 2020 is a testament to PTC's culture and its people. We strive to create a culture based on trust, respect, and inclusion."

PTC THERAPEUTICS 2020 CORPORATE RESPONSIBILITY

PTC's commitment to ESG focuses on five key areas: our **patients**, our **people**, our **community**, our **values** and the **environment**.

PTC'S COMMITMENT TO ESG FOCUSES ON FIVE KEY AREAS: OUR PATIENTS, OUR PEOPLE, OUR COMMUNITY, OUR VALUES AND THE ENVIRONMENT.

OUR PATIENTS

PTC was founded with the mission of discovering, developing and commercializing therapies for diseases with high unmet medical need. Today, we have multiple products on the market that are making a difference in the lives of patients and their families, and many more product candidates in our pipeline. Examples of our commitment to patients include:

- We continue to invest both internally and externally in cutting-edge research programs to search for treatments for patients suffering from diseases with little to no treatment options.
- We have doubled the number of scientists we employ over the last year and have significantly increased the number of internal research programs to search for drugs to treat rare diseases.
- We are also supporting external research programs for next generation rare disease therapies. We recently announced a regenerative medicine collaboration with the SMA Foundation to give grants to investigators exploring new approaches to promote muscle regeneration in patients with rare genetic disorders. This collaboration will fund six programs; three were funded in 2020 and three will be funded in 2021.
- We have made commitments to patients that they will continue to receive our investigational therapies beyond their clinical trial. For example, in the United States, we have been treating over 150 Duchenne muscular dystrophy patients with ataluren for free for over a decade.
- We have a long-standing commitment to work with and support patient advocacy groups, which are incredibly important assets to rare disease patients and their families. We have relationships globally with approximately 200 patient advocacy groups. We have committed significant funding through unsolicited grants to support patients through their patient advocacy groups.
- Over the last six years we have built a robust grant program called STRIVE for patient advocacy groups and have awarded over thirty grants to patient-focused groups through an independently governed review process.
  - An example of a recent recipient was the group called Cure Rare Disease, a patient organization based in Boston that will use the STRIVE funding to create a student life science immersion program that exposes school-age children to rare diseases to develop a greater sense of empathy for patients.
  - Other examples of recent STRIVE awards can be found at https://www.ptcbio.com/our-company/grants-and-donations/strive/
- We support programs to ensure patients in the United States can receive treatment despite insurance co-payment expenses. In 2020, PTC donated $2.2 million to organizations that assist patients with co-payment expenses.
- PTC has been a leader in providing diagnostic capabilities to healthcare professionals globally. We have invested in teaching physicians and healthcare providers to identify symptoms that are then diagnosed specifically – some of which can be treated by PTC with a current or future treatment option.

OUR PEOPLE

From our beginnings 23 years ago, and as a founder-led startup in New Jersey, we have grown to be a global company with over 1,000 employees. We take great pride in our corporate culture. Our employees share our sense of purpose and our goal of bringing life-changing therapies to patients in need. We support the growth of our employees in many ways, including:

- Regular use of the Gallup® Q12 survey to measure employee engagement, with a transparent process for discussing results and improving engagement. We work hard to ensure that employees are engaged.
  - In a recent pulse survey designed to assess our progress during the global pandemic, our scores (already above average for our industry) continued to increase even during the pandemic.
- Based on research, we evolved our management style to a coaching approach, focusing on employee strengths.

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KEY CORPORATE RESPONSIBILITY METRICS 2020

OUR PATIENTS

- **PTC invested 66%* in R&D** to continue to find and develop treatments for patients with unmet need
- To date, over **18,000 free genetic tests** have been distributed to support accurate diagnosis for rare disease patients
- **Partnership with over 200 global patient advocacy groups** to support patients with rare diseases
- Since 2015, **PTC has provided hundreds of millions of dollars of our treatments at no cost**
- **5 programs in place** to enable patients to be able to access PTC approved therapies **regardless of financial or insurance status** (PTC-ACTS+)
- Established program that allows siblings of our DMD patients to have **free access to our medicines**

OUR COVID-19 RESPONSE

- **Initiated Talented Pipeline Program** providing **53 recent graduates** on-the-job work experience through **one year internships**
- **Stopped all non-essential travel in February 2020** before the initiation of global lockdowns
- Established **3 COVID Taskforce teams**
  - -Team developing and monitoring safety protocols to **protect our workers** as well as business essential operations team focused on return to **long term planning**
  - -Team focused on **monitoring supply chain**, development and **commercial needs** during pandemic and transition to **remote working**
- **100% of research and tech facilities open** during the pandemic through optimized resource allocation
- Initiated clinical trial for a **potential COVID-19 treatment**

** As a percentage of Business Opearation Expenses (R&D + SG&A)*

OUR VALUES

- **Support local STEM programs** as part of our Adopt a School initiative
- **100% participation** in compliance training
- Formalized **ED&I program**; Hired Leader to **expand** program
- Chartered a woman's leadership group and black empowerment council
- Supported programs for the **advancement of underprivileged women**
- **$1.8M donated in 2020** through educational grants and donations

OUR PLANET

- **20% of our electricity** is from green sources
- **Installed charging stations** for electric cars at our Corporate Headquarters
- **Reduced radioactive waste** generation with new scientific approaches
- **36% of our total waste** was recycled
- Reduced landfill waste by **65 tons**

OUR PEOPLE

- **Increased our employee Gallup engagement scores** in every category; despite being in a pandemic
  - **96% of our employees** responded to the survey
- Increased **employee education opportunities by over 300%**
  - -Hosted **54** PTC University learning sessions covering topics from personal wellness to scientific discovery
- Global employee demographics
  - **48.5%** female
  - **45.5%** male
  - **6%** not declared
- Launched **Gallup's Clifton Strength Finders & HDBI** (Herrmann Brian Dominance Instrument) assessments with coaching to support employee development
  - **100% of employees** participated in Gallup Strength Finders
- Launched **Gallup Boss to Coach training** to mentor and support the development of our leaders
- **Free access** to a digital **on-demand career and management** learning solutions platform **for all employees**

- We use company-wide analytic and coaching tools to assess each employee's strengths.
- We utilize the Gallup® CliftonStrengths assessment and the Herrmann Brain Dominance Instrument (HBDI®) assessment to identify each person's top strengths and behavioral styles.

• We were awarded the Great Place to Work certificate in Brazil, our headquarters in Latin America.

• The sustained effort and success of these programs were recognized by the Gallup organization by receiving the 2021 "Don Clifton Strengths-Based Culture Award" from Gallup. PTC was one of six companies selected by an independent panel of judges to receive this award, which recognizes organizations with strong workplace cultures that get the best out of their employees.

• We have built a strong talent management program for leadership training throughout the organization. We also have built programs to groom high performing employees with training and mentorships to be the next generation company leaders.
- Our talent management program includes regular assessments by managers and peers, individual development plans at multiple levels, comprehensive succession planning, targeted retention programs, and a formal mentorship process to develop talented employees within the company.

• We have an overarching human resource business partner structure to implement these programs into practice. This represents both a financial investment in our workforce and a strong commitment to grow and develop our people.

• We have always had a strong culture of equity and diversity within the organization. In 2020 we formally established a global Equality, Diversity, and Inclusion (ED&I) program and named a Chief Culture & Community Officer. We also brought on board an experienced ED&I professional to spearhead our efforts.

• We have also recognized and appreciated the importance of being intentional about our diversity efforts. We have established multiple diversity groups within PTC that allow people to come together to discuss issues in a comfortable and safe setting. The ED&I groups meet regularly and interact with the CEO and Executive Committee. We have monthly programs to accentuate our diverse culture, each managed by a member of the Executive Committee.

• We have established a Global Outreach Steering Committee to focus on company-wide volunteer and giving opportunities. They focus on a strategy, identify volunteer opportunities, and target charities based on input from our employees.

• We offer employees an extensive range of educational benefits and opportunities, including a tuition reimbursement program, a company-wide education program ("PTC University") which showcases internal and external experts on a wide variety of topics, and support for membership in professional associations and attendance at educational conferences.

• We provide global training through a centralized learning management system for all employees tailored to their roles and responsibilities at PTC, including GxP training for appropriate employees.

• We manage our corporate goals and performance through the system of "OKRs," or "Objectives and Key results," pioneered at Intel by Andy Grove and widely used in the technology industry. All OKRs from the CEO on down are transparent to all employees through an internal system we developed and are refreshed and assessed on a quarterly basis.

• As PTC has been in a high growth mode for several years, we have developed an exceptional capability of attracting, recruiting, and onboarding talent globally, processes monitored by the Executive Committee.

• During the COVID-19 pandemic, PTC was one of the first companies to analyze public data on the virus and then reacted ahead of government guidance to ensure a safe and healthy environment. We established a Task Force with senior leaders which has been working 24/7 for more than a year to ensure appropriate onsite safety standards; we provide testing, conduct our own contact tracing, and now support the vaccine scheduling process. The result has been a safe and healthy work force both onsite and at home, with no interruptions to our business, and overall great business success.

OUR COMMUNITY

At PTC, we believe in paying it forward. On our journey from startup to a global commercial life sciences company, we overcame many obstacles. We have always given back to the communities in which we live and operate. We want to help others, both individuals and companies, see their own potential through our example. Some of the actions we are taking to further this goal are:

• We have launched a robust global internship program called the Talent Pipeline Program or TPP, during the COVID-19 pandemic to provide recent graduates real-world experience in the biopharmaceutical industry and related professions. TPP is a paid, one year-long program that provides on the job training, career planning and leadership development.
- Our recruiting included a focus on colleges that historically served the African American community in the United States as well as colleges for students from low-income families. We are proud of the diversity of the interns in this program.

• We work with local high schools in urban and underprivileged areas to expose them to the idea of a career in the life sciences. We support the Students2Science and the Passaic Academy of Science & Engineering programs, our scientists present at programs and career fairs in these schools, and we financially support these educational programs. We also support STEM and the life sciences programs through funding and other contributions and volunteer efforts by our employees.

• We believe in communicating all we are doing within our community, as exemplified by posts on social media and on our corporate website sharing our work with the larger community: https://www.ptcbio.com/news-resources/newsroom/

OUR VALUES

PTC knows our financial stakeholders expect us to conduct ourselves appropriately in terms of governance. In addition to having a robust Board of Directors that has access to the appropriate people and resources to oversee the Company, this includes assessing risks and working to ensure ethical behavior by our employees and business partners globally. Examples of our commitments in this area include:

• We have a formal Board committee structure for oversight and risk management, including independent chair and CEO roles. Since our initial public offering in 2013, we have added four new Board members and propose to add a fifth this year. If the directors proposed for election this year receive approval from our shareholders, PTC's Board will meet the proposed NASDAQ requirements for Board diversity and will include four women (on a Board of 10 directors).

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ESG Continued

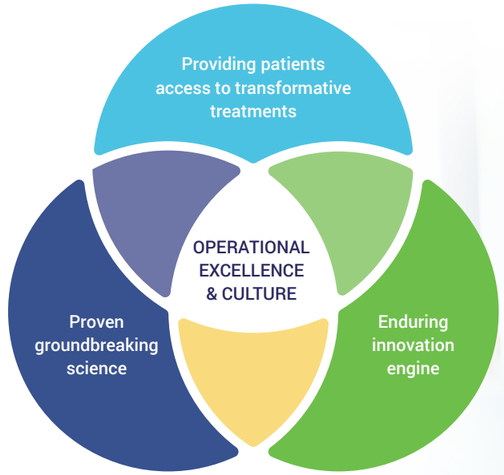
- We have a fully developed Global Compliance Program that incorporates the elements of an effective compliance program in accordance with the "Compliance Program Guidance for Pharmaceutical Manufacturers," developed by the United States Department of Health and Human Services, Office of Inspector General ("OIG") and other laws and regulations that govern our industry. Key aspects of our program include written policies and procedures, general and role specific-company-wide training, training of business partners, regular auditing and monitoring, third party due diligence and where necessary disciplinary actions and corrective measures.
- Our Chief Compliance Officer manages our compliance program with a global team, including representatives in Europe and Latin America, and has direct access and dotted line reporting to our CEO and Board members.
- Our Code of Business Conduct and Ethics and related compliance policies and procedures are prominently posted on our Company Intranet. Select policies and procedures are available to the public via our corporate website: https://www.ptcbio.com/our-company/about/global-compliance-program/.
- Our employees have access to multiple channels to report areas of concern, including our confidential whistleblower hotline via an external vendor.
- We routinely conduct audits of our global offices and our global partners, either using our internal compliance audit team or our external audit partner (a big four accounting firm).
- We employ a formal diligence process to assess potential new distributors and other key business partners, including use of an external vendor for background checks.
- Our global distribution partners are required to have in place or adopt an anti-corruption policy and related training and audit obligations as part of our standard agreement form.

ENVIRONMENT

As a science-based company, we understand the impact people have on the environment. We as well as our employees care about the world we live in and have a stead-fast commitment to maintaining the environment. We have always ensured our actions were compliant with environmental requirements and regulations and have encouraged employee actions which are environmentally friendly.

- Our laboratories meet all environmental standards and have consistently passed inspections by multiple government authorities.
- We have installed charging stations for electric vehicles at our corporate headquarters.
- We have replaced lightbulbs in all buildings at our corporate headquarters with LED lighting.
- We have utilized innovative scientific approaches to reduce generation of radioactive waste.
- 36% percent of PTC's total waste in 2020 was recycled, reducing landfill waste by 65 tons.
- We have a strong company-wide recycling program and seek to maximize our use of recycled materials.
- We have filtered water coolers in all facilities to encourage the use of tap water in lieu of using bottled water.
- We encourage our employees to use reusable water bottles and cups, plates and silverware to reduce the use of paper or plastic cups.
- We have replaced older air handlers with more energy efficient units to utilize non-CFC refrigerants.
- We have incorporated Hazardous Waste Minimization procedures in our laboratory operations.
- We have organized a "Green Team" committee that takes action through recycling and reduction of food waste programs, as well as other environmentally sound programs.

PTC has built a strong, sustainable company to execute on our mission



PTC Expectations

EVER BETTER
We are always raising the bar. We act with a focus on quality and a sense of urgency.

PASSIONATE ABOUT PURPOSE
We are more than a company; we work for a cause. We make decisions based on patients' needs.

CHAMPION INCLUSION, TRUST & RESPECT
We leverage the strength of our diversity. We require a fair and ethical environment where employees can flourish.

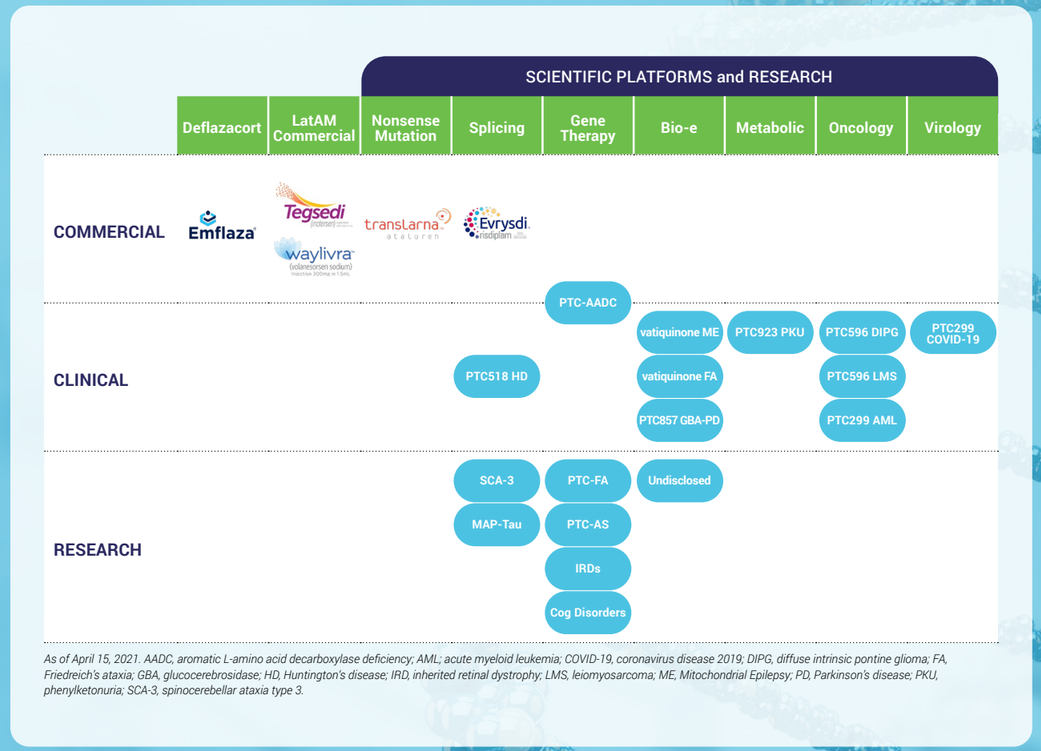
BE BOLD
We challenge. We adapt.

WORK AS ONE PTC
We are a dynamic, global network of empowered, high performing teams that achieve extraordinary results. We partner openly to bring out the best in ourselves and maximize talent.

THINK LIKE AN ENTREPRENEUR
We are energized by solving difficult problems, bringing innovation & creativity to our work. We are tenacious & tireless in our quest for breakthrough solutions.

BE KIND
We go beyond just being nice. We are friendly, considerate and helpful. We care about each other.

MAKING GREAT progress



As of April 15, 2021. AADC, aromatic L-amino acid decarboxylase deficiency; AML; acute myeloid leukemia; COVID-19, coronavirus disease 2019; DIPG, diffuse intrinsic pontine glioma; FA, Friedreich's ataxia; GBA, glucocerebrosidase; HD, Huntington's disease; IRD, inherited retinal dystrophy; LMS, leiomyosarcoma; ME, Mitochondrial Epilepsy; PD, Parkinson's disease; PKU, phenylketonuria; SCA-3, spinocerebellar ataxia type 3.

INAUGURAL AADC DEFICIENCY AWARENESS DAY

On October 23, 2020, PTC was honored to present the AADC Family Network-sponsored inaugural AADC Deficiency Awareness Day for the Commonwealth of Massachusetts. We broadcasted a live discussion of this official designation on the PTC Therapeutics Facebook channel (facebook.com/ptctherapeutics). This event successfully achieved more than one million impressions on social media channels, helping to raise awareness of this rare disease, and to enable caregivers, health care professionals and patient advocates in sharing resources. The live broadcast featured presentations by then MassBio President & CEO Bob Coughlin, Boston Children's Hospital neurologist Irina Anselm, MD, and PTC's Chief Development Officer, Matthew B. Klein, MD, MS FACS. The featured speaker was Kelly Heger, founder of the AADC Family Network and an AADC deficiency parent herself. Kelly's heartfelt recounting of her family's experience raising Jillian, a young woman with AADC deficiency, received overwhelming encouragement from public comments on Facebook, during and since the broadcast.

This event was coordinated by members of PTC's Government Relations, Patient Engagement and Corporate Communications teams.

"We are endlessly inspired by the strength and courage exhibited by the Heger Family – and by the love they show Jillian," explains project leader Ted Piper. "Our team channeled this inspiration into an amazing example of teamwork and collaboration to help Kelly mark an awareness day and raise awareness of AADC deficiency across the globe."

STRIVE AWARDS PROGRAM

2020 marked the 6th year of PTC's STRIVE (Strategies to Realize Innovation, Vision and Empowerment) Awards Program for Duchenne muscular dystrophy.

The STRIVE program provides grants to patient advocacy organizations globally to realize meaningful projects that address the unmet needs of the rare disease community. Since its launch in 2015, the STRIVE Award has supported **32 patient groups** whose programs have made a positive impact through increased awareness or diagnosis of Duchenne, advanced education, improved quality of life of patients, improved patient access to medical care or fostering of future patient advocates.

With the unprecedented events of this year the role of patient organizations has been more vital than ever in providing support and keeping rare disease communities connected.

In 2020, we were proud to recognize four winning initiatives and communities who have continued to show uninterrupted resilience for the Duchenne community around the world:

- **Argentina: Associacion Distrofia Muscular**
  Project that includes an online platform to improve peer-to-peer connections
- **Hungary: The Healing Goodwill Foundation**
  Project that involves a series of events designed to connect Duchenne families with healthcare professionals
- **Russia: GAOORDI**
  'Not Alone' program that will support families navigate the emotional journey of living a rare disease diagnosis
- **United States: Cure Rare Disease**
  Ambassadors Program 'Student Education & Life Science Immersion Program' that will raise awareness of Duchenne in schools

To learn more about the winning organizations and their initiatives, please click here to watch the video.

"I am so proud of the continued support we are able to provide to these organizations all around the world through the STRIVE Awards, so that they can keep Duchenne communities supported and connected through this turbulent time," said Mary Frances Harmon, Senior Vice President, Corporate Relations, PTC Therapeutics.

GLOSSARY

AADC: AADC Deficiency (AADC-d) is a rare central nervous system disorder arising from reductions in the enzyme aromatic L-amino acid decarboxylase (AADC) that result from mutations in the dopa decarboxylase (DDC) gene. This reduction leads to deficits in the neurotransmitters dopamine, norepinephrine, epinephrine, serotonin and melatonin. AADC Deficiency causes severe developmental delays, the inability to develop any motor strength and control (global muscular hypotonia/dystonia) resulting in breathing, feeding, and swallowing problems, frequent hospitalizations, and the need for life-long care. Patients with severe forms often die in the first decade of life due to profound motor dysfunction, autonomic abnormalities, and secondary complications such as choking, hypoxia, and pneumonia. No treatment options other than palliative care currently exist for many AADC patients.

AML: Acute myeloid leukemia (AML) is a cancer characterized by the rapid growth of abnormal cells that build up in the bone marrow and blood and interfere with normal blood cells. Symptoms may include feeling tired, shortness of breath, easy bruising and bleeding and increased risk of infection. Occasionally, spread may occur to the brain, skin or gums. AML progresses rapidly and is typically fatal within weeks or months if left untreated.

AS: Angelman Syndrome (AS) is a severe neurological development disorder characterized by profound developmental delays, problems with motor coordination (ataxia) and balance, and epilepsy. Individuals with AS do not develop functional speech, have seizures and sleeping difficulties. AS is caused by a problem with UBE3a gene and affects all races and both genders equally. People living with AS require life-long care, intense therapies to help develop functional skills and improve their quality of life, and close medical supervision involving multiple interventions. AS may be misdiagnosed since other syndromes have similar characteristics. There are currently no approved treatments for AS.

DIPG: Diffuse interstitial pontine glioma (DIPG) is a rare, rapidly fatal pediatric brain tumor. Patients are usually diagnosed between 5-6 years of age. 98% of patients die within two years of diagnosis.

DMD: Duchenne muscular dystrophy (DMD) is the most common and one of the most severe types of muscular dystrophy. DMD occurs when a mutation in the dystrophin gene prevents the cell from making a functional dystrophin protein. Dystrophin is a muscle membrane associated protein and is critical to the structural and membrane stability of muscle fibers in the skeletal, diaphragm and heart. The absence of normally functioning dystrophin results in muscle fragility, such that muscle injury occurs when muscles contract or stretch during normal use. As muscle damage progresses, connective tissue and fat replace muscle fibers, resulting in inexorable muscle weakness. Patients with DMD typically lose walking ability by their early teens, require ventilation support in their late teens and, eventually, die due to heart and lung failure. The average age of death for DMD patients is in their mid-twenties.

GBA Parkinsons: GBA-Parkinson's disease (GBA-PD) occurs as a result of a mutation in the GBA gene, which makes the glucoscerebrosidase enzyme. Deficits in this enzyme correlate with motor symptom dysfunction, cognitive decline, and diminishing gait and balance.

FA: Friedreich's ataxia (FA) is an inherited neuromuscular disorder most commonly caused by a single genetic defect in the FXN gene that leads to reduced production of frataxin, a mitochondrial protein that is important for cellular metabolism and energy production. FA results in a physically debilitating, life-shortening condition and is the most common hereditary ataxia. Symptoms of FA include progressive loss of coordination and muscle strength, which lead to the full-time use of a wheelchair; scoliosis (which often requires surgical intervention); diabetes mellitus; hearing and vision impairment; serious heart conditions; and premature death. Current FA therapies are primarily focused on symptom relief, and there are no FDA-approved drugs to treat the cause of FA.

FCS: Familial Chylomicronemia Syndrome (FCS) is an ultra-rare disease caused by impaired function of the enzyme lipoprotein lipase (LPL) and characterized by severe hypertriglyceridemia (>880mg/dL) and a risk of unpredictable and potentially fatal acute pancreatitis. Because of limited LPL function, people with FCS cannot breakdown chylomicrons, lipoprotein particles that are 90% triglycerides. FCS patients are also at risk of chronic complications due to permanent organ damage. They can experience daily symptoms including abdominal pain, generalized fatigue and impaired cognitions that affect their ability to work. People with FCS report major emotional and psychosocial effects including anxiety, social withdrawal, depression and brain fog. There is no effective therapy for FCS currently available.

hATTR: hereditary transthyretin (hATTR) amyloidosis is a progressive, systemic and fatal inherited disease caused by the abnormal formation of the TTR protein and aggregation of TTR amyloid deposits in various tissues and organs throughout the body, including in peripheral nerves, heart, intestinal tract, eyes, kidneys, central nervous system, thyroid and bone marrow. The progressive accumulation of TTR amyloid deposits in these tissues and organs leads to sensory, motor and autonomic dysfunction often having debilitating effects on multiple aspects of a patient's life. Ultimately, hATTR amyloidosis results in death within three to 15 years of symptom onset. Therapeutic options for the treatment of patients with hATTR amyloidosis are limited.

HD: Huntington's Disease (HD) is a rare genetic disorder that is caused by a CAG repeat expansion in the HTT gene. The mutated HTT protein leads to severe neuron degeneration predominately in the striatum and the cerebral cortex. Currently, there are no approved disease-modifying treatments.

IRDs: Inherited retinal disorders are a group of rare eye disorders caused by an inherited gene mutation and can result in vision loss or blindness. Some people with inherited retinal diseases experience a gradual loss of vision, eventually leading to complete blindness. Others may be born with or experience vision loss in infancy or early childhood.

LMS: Leiomyosarcomas (LMS) are malignant tumors of muscle tissue. They are rare tumors with a high rate of relapse. Median overall survival is 14 months.

MEDS: Mitochondrial Epilepsy Disorders (MEDS) are part of a group of conditions called, metabolic disorders. The organs with the most mitochondria in them are the brain, nerves, muscles and liver and because of this, neurological disorders, including epilepsy, occur quite commonly in mitochondrial disorders. Most of the epilepsy caused by a mitochondrial disorder starts in childhood and usually in the first two years of life. Most mitochondrial disorders are progressive meaning the symptoms and the seizures will worsen over time. How quickly the progression happens will depend on the particular type of mitochondrial disorder. The seizures in most mitochondrial disorders are usually very difficult to control. Unfortunately, for most mitochondrial disorders there is no specific treatment, such as diet or surgery, which can stop the seizures or stop the disorder from progressing.

PKU: Phenylketonuria (PKU) is a rare inherited metabolic disorder and is caused by a defect in the gene that helps create the enzyme needed to break down phenylalanine. Without treatment, phenylalanine can build up to harmful levels in the body, causing mental retardation, cognitive disabilities, seizures and other serious problems. The majority of patients do not initially respond or are not well controlled by the standard of care.

SMA: Spinal Muscular Atrophy (SMA) is a genetic disease caused by mutation or deletion of the SMN1 (survival of motor neuron) gene. In its most severe forms, is associated with a high rate of childhood mortality. SMA is characterized by progressive loss of motor neurons, muscle weakness, and atrophy. The disease affects mainly proximal muscles including intercostal muscles (chest muscles), and patients often die due to respiratory complications.

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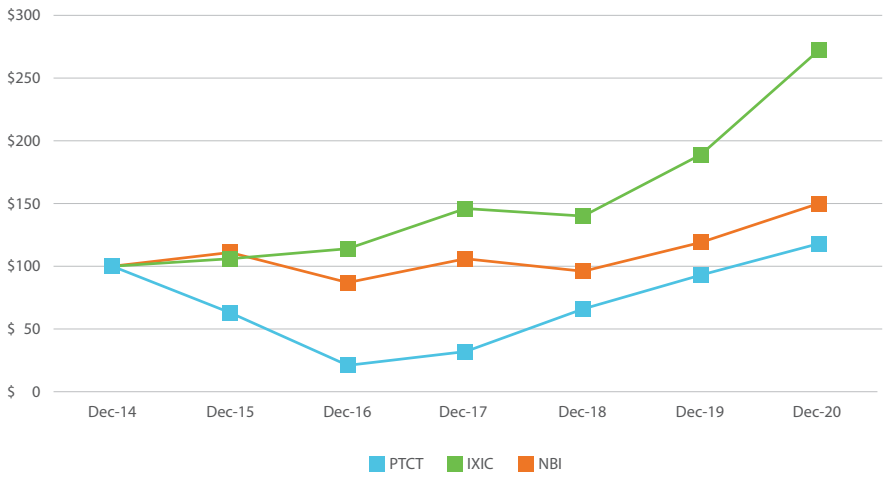
STOCK PERFORMANCE GRAPH*

The following graph illustrates a comparison of the total cumulative stockholder return on the Common Stock of PTC Therapeutics' Stock from investing on January 1, 2014 through December 31, 2020, in two indices: the NASDAQ Biotechnology Index (NBI) and the NASDAQ Composite Index (IXIC). Data for the NASDAQ Biotechnology Index (NBI) and the NASDAQ Composite Index (IXIC) assume reinvestment of dividends. The stockholder return shown in the graph below is not necessarily indicative of future performance, and we do not make or endorse any predictions as to future stockholder returns.



**The information contained in this Stock Performance Graph shall not be deemed "soliciting material" or to be "filed" with the SEC, for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or the Exchange Act, or otherwise subject to the liabilities under that Section, and shall not be deemed to be incorporated by reference into any filing of under the Securities Act of 1933 or Securities Exchange Act of 1934, each as amended, except to the extent that we specifically incorporate it by reference into such filing.*

$100 Investment in Stock or Index	Dec 31, 2014	Dec 31, 2015	Dec 31, 2016	Dec 31, 2017	Dec 31, 2018	Dec 31, 2019	Dec 31, 2020
PTC Therapeutics, Inc. (PTCT)	$ 100	$ 63	$ 21	$ 32	$ 66	$ 93	$ 118
NASDAQ Composite (IXIC)	$ 100	$ 106	$ 114	$ 146	$ 140	$ 189	$ 272
NASDAQ Biotechnology Index (NBI)	$ 100	$ 111	$ 87	$ 106	$ 95	$ 119	$ 150

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STOCKHOLDER INFORMATION

Market Information
PTC's common stock trades on the NASDAQ Global Market under the ticker symbol PTCT.

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Annual Meeting
The Annual Meeting of the Stockholders will be held on Wed., June 8th at 9am. Due to uncertainties of the novel Covid-19 pandemic and health restrictions, please check the company website for the location 2 weeks before the meeting.

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PTC 2020 Annual Report