

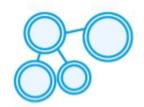


## January/February 2021 Research Roundup

Our Research Support Network (RSN) connects you to Parkinson's research. From finding out more about research to getting involved, there's something for everyone. You can register to receive our research emails directly by visiting

www.parkinsons.org.uk/research/get-involved-research and clicking on "Join the Network."

Here's our **January/February Research Roundup** with the latest research news and opportunities for you.



## **GET CONNECTED**

## Turning the tide on big pharma investment

We explore the crucial role pharmaceutical companies play in delivering new treatments and take a look at some recent major investments to drive forward new therapies for Parkinson's.

As we've seen this past year with the rapid development of Covid-19 vaccines, large pharmaceutical companies are still crucial players in developing and delivering new medicines. Research is a team effort, but like a star striker in football, large pharma are usually the ones that are needed to get the ball (or new treatment) over the line. These multinational companies have the expertise and resources to conduct large-scale global clinical trials and deliver new medicines that can be made available on a global scale.

There are many conditions that need cures and they are all in need of the investment that pharmaceutical companies bring. So what makes a company invest in one condition over another?

As businesses, clearly a major driving factor is the potential market size: how many people need (and could pay for) this medicine? With Covid, the unprecedented market size fuelled the race for vaccines. Outside of Covid, conditions that affect the most people tend to be near the top of the list, and <u>cancer sits at the top of the pile</u>.

For conditions like Parkinson's, the dedicated focus and investment of pharmaceutical companies is harder to come by. Parkinson's affects millions globally and there is a huge need for better treatments, especially those that can slow, stop or reverse the condition — so pharma are

definitely still interested. But they are cautious. Neurodegenerative conditions are especially difficult to develop treatments for, partly due to their complexity and partly because it's hard to get drugs into the brain where they're needed.

For these reasons, pharma companies have retreated from investing in the early stages of drug development for conditions like Parkinson's, leaving a gap in the drug development pipeline. This has led to the rise of smaller, nimbler companies called biotechs that have stepped in to fill this gap. These smaller companies don't have the money to take a new treatment all the way, but by investing in the difficult and risky early stages of drug development they can take projects far enough to attract a larger company's attention. If successful, a larger company will swoop in to invest to take their therapy forwards into the expensive later stages of development including clinical trials.

This approach inspired <u>Parkinson's UK to launch its own Parkinson's Virtual Biotech programme in 2017</u>. Unlike real biotech companies, we're not in it for profit. Our sole mission is to accelerate the development of new Parkinson's treatments and 'de-risk' projects to entice pharmaceutical companies to bring their considerable funds to the later stages of development. And now, with a growing portfolio of promising projects, and with the investments we've seen large pharma making this year, we're confident that we're on the right track.

#### Major investments in 2020

Let's review some of the deals we've seen struck in 2020.

Eli Lilly makes \$1billion deal with Prevail Therapeutics to advance GBA therapies for Parkinson's. Prevail Therapeutics has been investing in developing treatments that tackle a specific type of genetic Parkinson's linked to changes in the GBA gene since 2017. The company now has two potential drugs of interest and its frontrunner— PR001 — is entering phase 1/2 clinical trials for Parkinson's and Gaucher's disease (a condition also caused by GBA changes).

In a deal worth up to \$4billion, German company Bayer acquired gene therapy biotech AskBio. One of AskBio's most advanced experimental therapies is a gene therapy approach, developed by Brain Neurotherapy Bio, to deliver glial cell-derived neurotrophic factor (GDNF) for Parkinson's, which is currently in a Phase 1b clinical study in the United States. You might be familiar with GDNF and the challenges of delivering this protein into the brain. With gene therapy, it might be possible to use an engineered virus to deliver the instructions for cells to make their own GDNF. This would mean that the brain can continue to make its own supply of this potentially restorative protein.

And this is not the only industry programme focused on developing new treatments using neurotrophic factors. Recently two Finnish companies — Nanoform and Herantis Pharma have joined forces to progress a therapy for Parkinson's that can deliver CDNF (a very similar protein to GDNF) using a pioneering nasal delivery approach.

Biogen and Denali announced a \$1billion collaboration to progress drugs targeting LRRK2. Specific changes in a gene called LRRK2 are linked to an increased risk of Parkinson's and therefore treatments that can target LRRK2 hold hope as a cutting-edge new treatment. Denali Therapeutics have developed an exciting drug candidate called DNL151 which aims to reduce

the activity of LRRK2. The drug has already been tested in small, early stage trials in people with Parkinson's and appears to be safe. Now major pharma company Biogen is investing to take DNL151 into larger trials, expected to start in 2021, which will explore whether it could slow the development of Parkinson's.

LRRK2 inhibitors are not the only new therapy Biogen is investing in. As we move into 2021, we will be looking out for results from a clinical trial testing <u>Biogen's BIIB054 (cinpanemab)</u> anti-alpha synuclein antibody. This approach focuses on trying to clear away a protein called alpha-synuclein which is thought to be a driving factor in the development of Parkinson's and early results showed promise.

Finally, we will also be keeping an eye out for results from the second part of the phase 2 study of Roche and Prothena's prasinezumab — a vaccine like approach to clearing alpha-synuclein in Parkinson's. The results after 1 year were mixed but the trial has continued into its second year and is gathering more important data on this potential new treatment.

#### Keep up with research in 2021 and beyond

There is certainly a lot to look out for in 2021 and beyond, and with so many players, both new and old, willing to invest in the next generation of Parkinson's treatments, the future is looking bright.

<u>Check out research advocate, Kevin McFarthing's blog on his work mapping the clinical trial landscape in Parkinson's.</u>

There are hundreds of trials testing new Parkinson's treatments underway around the world so keep your finger on the pulse by joining our Research Support Network. We'll send you email updates on all the latest news and tell you about opportunities to take part and shape research to drive forward better treatments and a cure.

Written by Dr Beckie Port, Research Communications Manager at Parkinson's UK

For more research articles, please visit <a href="https://medium.com/parkinsons-uk">https://medium.com/parkinsons-uk</a>

## Catch up on recorded research events

- 'Parkinson's and Technology' hosted by the <u>Dundee Research Interest Group</u>, featuring talks on:
  - 'Time to change: the role of digital technology in the future of PD healthcare' with speaker Lynn Rochester (at 14:46 in video)
  - 'Rehabilitation technology to support mobility in Parkinson's' with speaker Andy Kerr (at 46:32)
  - 'Model-based treatment for Parkinson's' with speaker Ray Finucane (at 1:15:40)
  - o 'Democratising Healthcare Technologies: Wearables for People living with

Available to watch online here: <a href="https://www.youtube.com/watch?v=ujTIL09\_-mo">https://www.youtube.com/watch?v=ujTIL09\_-mo</a>

• 'Meet the 'researched' - Not just a load of old tablets' hosted by the Peninsula Parkinson's Research Interest Group. They turn the Zoom camera on the other end of the stick, the people who participate in research as the target of a novel treatment. Drs. John and Sue Whipps give a presentation about their journey from his diagnosis to their involvement in clinical trials, explaining the issues they dealt with along the way. They take questions from a Panel and explore the challenge of finding and motivating people to get involved.

#### Available to watch online here:

https://www.youtube.com/watch?v=qjhk738UOrl&feature=youtu.be

You can find more research event recordings online here:

https://www.parkinsons.org.uk/research/research-events

# You're invited to an online research event: Parkinson's and Technology, part 2

When: Saturday 27 February 2021, 2:00pm to 4:45pm

Where: Online via Zoom

The <u>Dundee Research Interest Group</u>, in collaboration with the <u>Special Interest Group for Parkinson's and Technology</u>, warmly invite you to the second in a planned series of online research events focused on intelligent apps, devices and solutions to support people affected by Parkinson's (part one available to watch above). The focus of this event is tools to manage everyday tasks that can become challenging due to the symptoms of Parkinson's. Three presentations will address this topic:

- Dr Katherine Fletcher, Parkinson's UK's Research Communications Manager, talks about non-drug therapies and devices.
- Professor Wan-Fai Ng, University of Newcastle, shows ways to measure and manage fatique.
- Julie Dodd, Parkinson's UK's Director of Transformation, describes the potential of apps for people with Parkinson's and their limitations.

For more information and to register for your free place, please visit the Eventbrite page.



For people who wish to participate in studies, please visit our **Take Part Hub**, a post code searchable database of studies actively recruiting participants. The Hub is updated weekly with new studies, so please do check it regularly:

https://www.parkinsons.org.uk/research/take-part-research

And for those people not online, you can call our free, confidential Helpline on 0808 800 0303 and our trained Advisors will be able to discuss what you are interested in and put you through to the Research team to find studies for you.

### PD Frontline - Identifying genes in people with Parkinson's

The aim of the research is to identify a group of people who have changes in specific genes so they can be invited to take part in future research trials. The design of research trials is changing and knowing people's genetic sequencing may help personalise and improve research.

Who is needed: 1000 people diagnosed with Parkinson's who live anywhere in the UK.

**What is involved**: You will need to register on the PD Frontline website where you will be asked to complete an online consent form and a short 10-minute survey. Upon completion you will be sent a saliva sample collection kit which will be posted to you with return pre-paid packaging.

For full information about the research and to register: Please go to the PD Frontline website <a href="https://pdfrontline.com/en">https://pdfrontline.com/en</a>

If you have problems accessing the website please contact the research team via email (pdfrontline@ucl.ac.uk) or phone (0208 016 8431).

**Need to chat to someone?** Our helpline and Parkinson's local advisers are here to answer any questions you have about the symptoms of Parkinson's. You can call them on **0808 800 0303**.

Thank you for supporting research!
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