

Building a healthier future

# Cell Therapy & Gene Therapy



## Parkinson's Disease

### What is Parkinson's disease?

Parkinson's disease, or PD for short, is a chronic, progressive disease of the nervous system marked by the degeneration of cells that produce the neurotransmitter dopamine — a chemical messenger responsible for regulating body movements, among other functions.

PD can manifest with unspecific symptoms such as sleep disorders, loss of smell, or depression long before the typical PD symptoms such as an uncontrollable tremor of the hand or a generalized stiffness in body parts appear. With time, symptoms worsen, making simple tasks like climbing stairs, writing a letter, or eating and drinking very challenging and heavily impacting quality of life. In late stages, dementia, bladder problems and profound fatigue next to other psychiatric problems can develop.

Four characteristic signs and symptoms of PD can be remembered by the following acronym, TRAP<sup>1</sup>

**T**

**Tremor:**

Shaking, often in arms, legs, lips, or jaw<sup>1</sup>

**R**

**Rigidity:**

Stiffness of the limbs, neck, or trunk<sup>2</sup>

**A**

**Akinesia/bradykinesia:**

Impaired voluntary movement<sup>1</sup>

**P**

**Postural instability<sup>1</sup>**

Symptoms and progression vary from person to person.



PD is a very common neurodegenerative movement disorder<sup>3</sup>, affecting over 10 million people worldwide<sup>4</sup>. Did you know that men are 50% more likely to develop PD than women<sup>5</sup>, and in most people, symptoms first appear around the age of 60<sup>6</sup>?

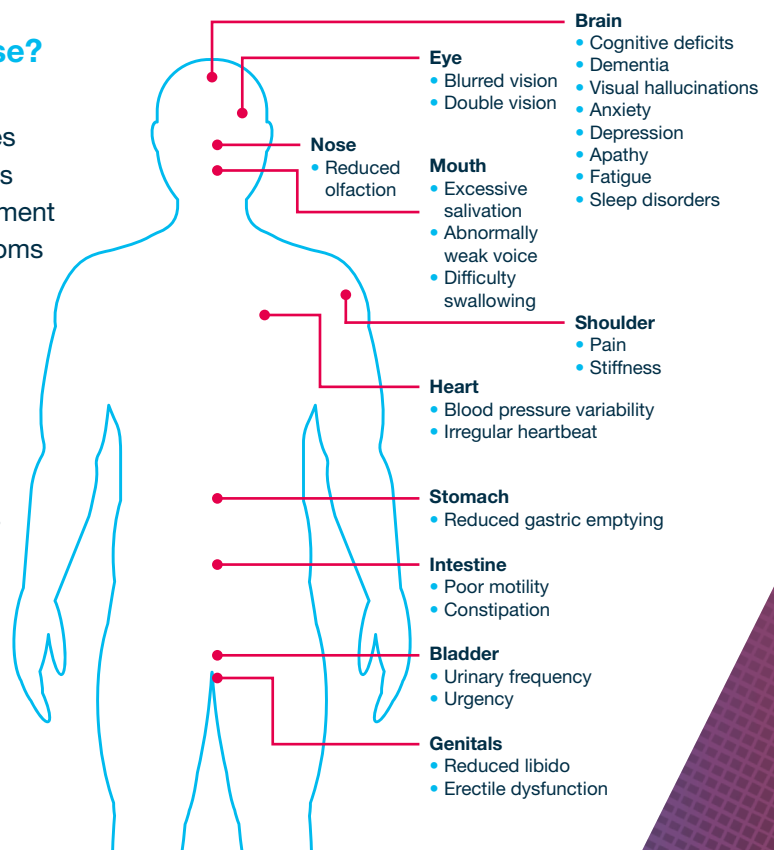
### Is there a treatment for Parkinson's disease?

The most widespread current treatment for PD focuses on replacing dopamine with oral medication. While this eases symptoms at the beginning, over time the treatment becomes less and less effective, meaning that symptoms come back, and patients get worse. This is because the root cause of the disease, the irreversible loss of dopamine-producing neurons, is not addressed.

In addition to how the disease affects quality of life of people living with it as well as their carepartners and loved ones, many of the costs associated with the disease also present an economic burden for patients and their families.

#### The estimated costs of treating PD:

- €13.9 bn estimated in Europe<sup>7</sup>
- \$26.5 bn estimated in US<sup>8</sup>





## What if we could use cell therapy or gene therapy to tackle Parkinson's disease?

Bayer is, together with its fully owned subsidiaries Asklepios BioPharmaceutical (AskBio) and BlueRock Therapeutics, committed to developing cell therapies and gene therapies for the treatment of PD. Two assets are currently being evaluated in clinical development, and both candidates aim to change the disease progression after a one-time treatment by restoring and boosting lost dopamine activity, which could improve symptoms experienced and quality of life for patients long-term.



### Cell Therapy

We're working on a PD treatment to replace lost dopamine-producing neurons in the brain with new ones.

BlueRock is advancing a cell therapy candidate consisting of pluripotent stem cell (PSC)-derived dopaminergic precursor cells. This approach aims to reinnervate the brain, and restore lost dopaminergic function.



### Gene Therapy

We're testing a treatment that could prompt the brain's own cellular machinery to reinvigorate nerve cells that degenerate due to PD.

AskBio is working on a gene therapy that aims to upregulate a naturally occurring gene into the brain, which is responsible for the expression of a protein called GDNF (glial cell-derived neurotrophic factor). This protein supports the survival and stimulation of the dopamine-producing neurons which are progressively dying.



AskBio and BlueRock's clinical candidates are investigational therapies that have not received marketing authorization by any regulatory authority, and their efficacy and safety have not been established or fully evaluated.

## Bayer's commitment to helping Parkinson's patients and beyond

Cell therapies and gene therapies are geared to transform medicine. Already today they positively impact the lives of patients living with often life-threatening diseases. And there are good reasons to be excited, as several clinical programs continue to present promising results in humans.

Together with AskBio and BlueRock, we are committed to transforming the potential of cell therapy and gene therapy into benefit for people living with conditions like Parkinson's disease.



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