
NECTAR
NECTAR 2023
33rd Annual Meeting of the
Network for European
CNS Transplantation and Restoration
Naples, Italy • 23-25 October 2023

The Organising Committee

Supporters

Aspen Neuroscience

BioLamina — Revolutionizing Cell Culture

BlueRock Therapeutics

Campaign for Alzheimer's Research Europe

CURE Parkinson's

Neuratris — Innovation for Translational Neuroscience

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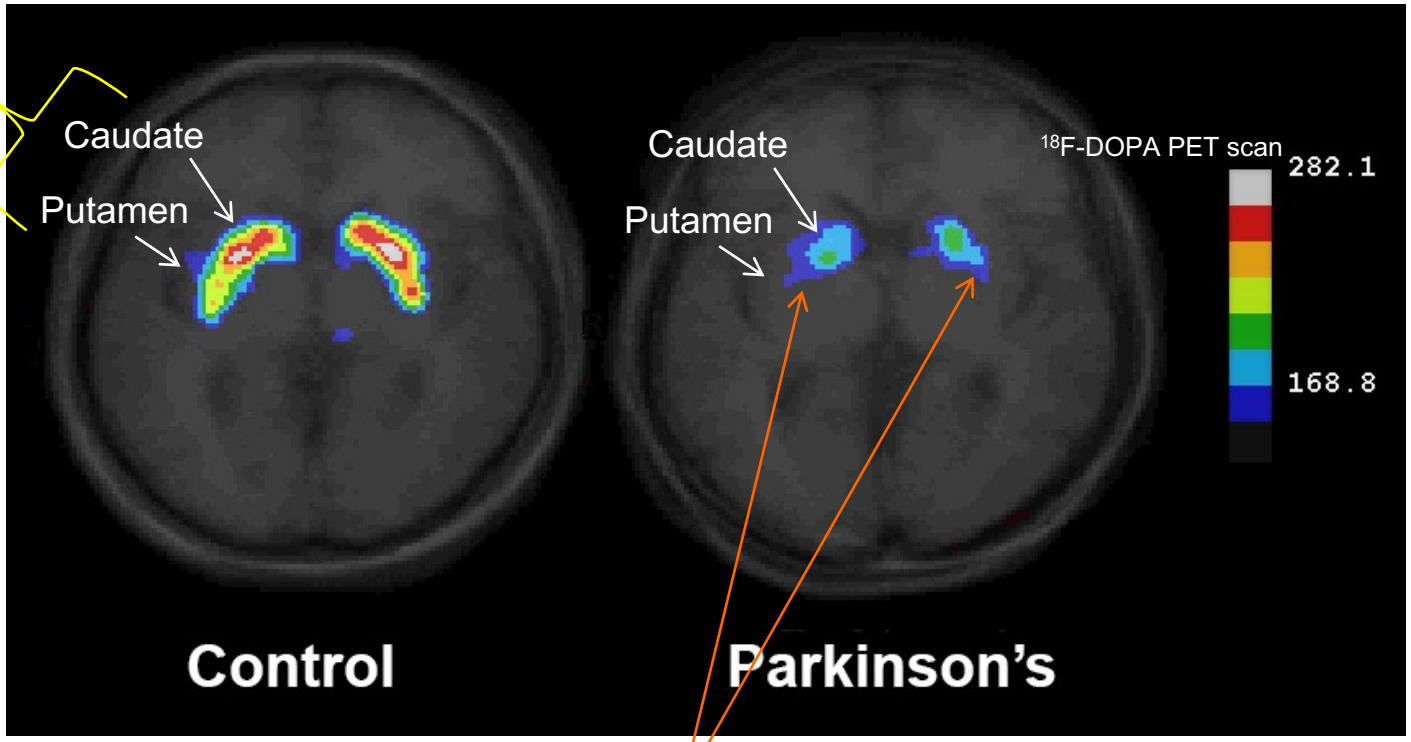
TreeFrog therapeutics

VectorBuilder — Revolutionize Gene Delivery

Cell replacement therapy (CRT) for Parkinson's

^{18}F-DOPA PET scan

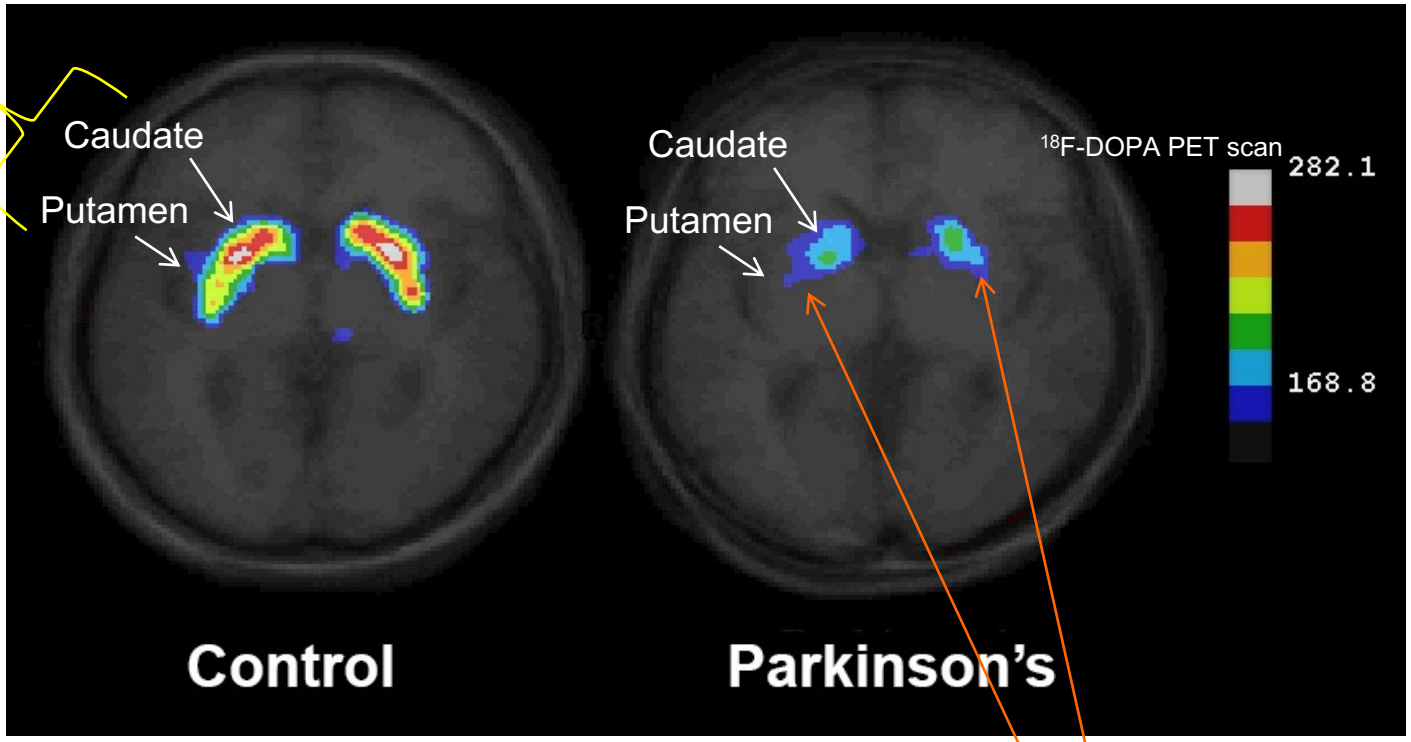


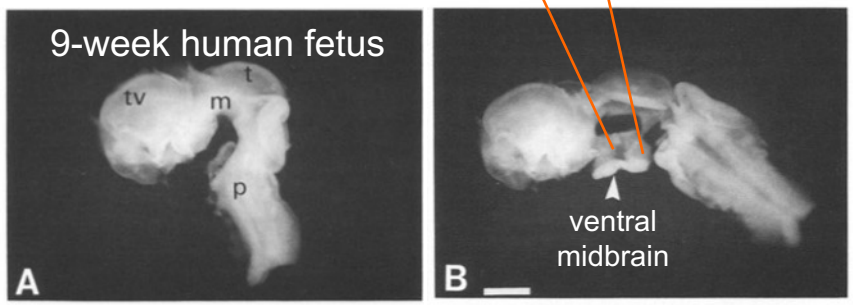
- Motor symptoms caused by loss of **dopaminergic neurons**
- The loss is very localised to the **caudate** and **putamen**

Therapy: Transplant ***new*** dopaminergic neurons into putamen

Cell replacement therapy (CRT) for Parkinson's

^{18}F-DOPA PET scan





(Brundin *et al*, 1986, *Exp Brain Res*)

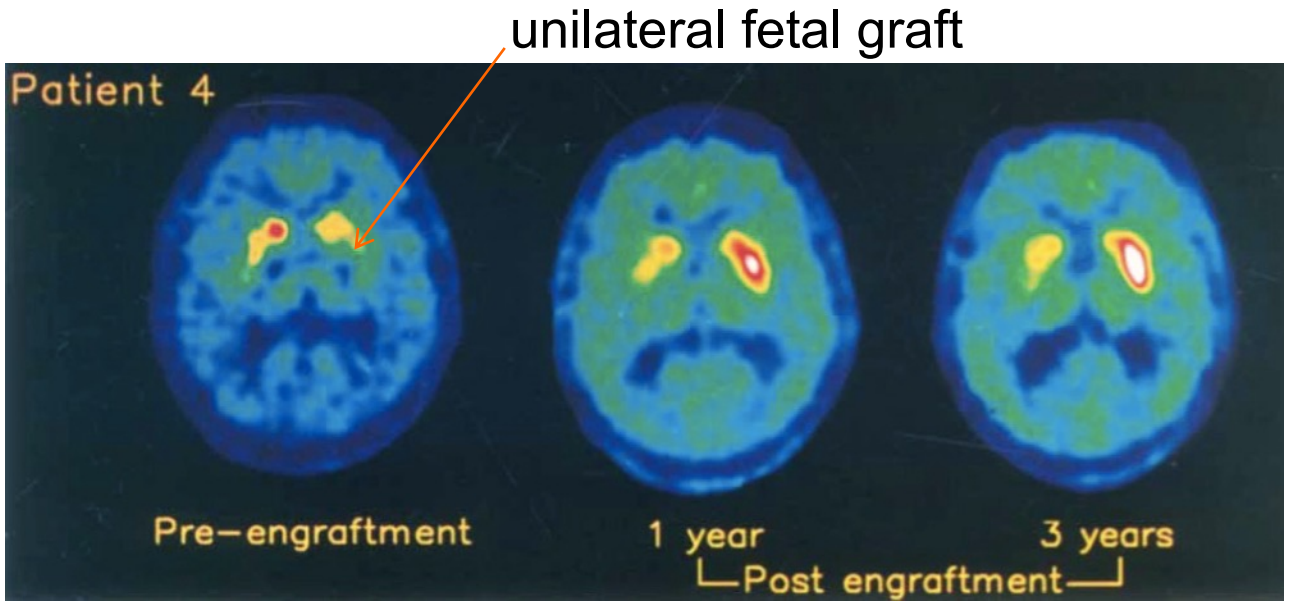
- Motor symptoms caused by loss of **dopaminergic neurons**
- The loss is very localised to the **caudate** and **putamen**

Late 1970s

Olle Lindvall

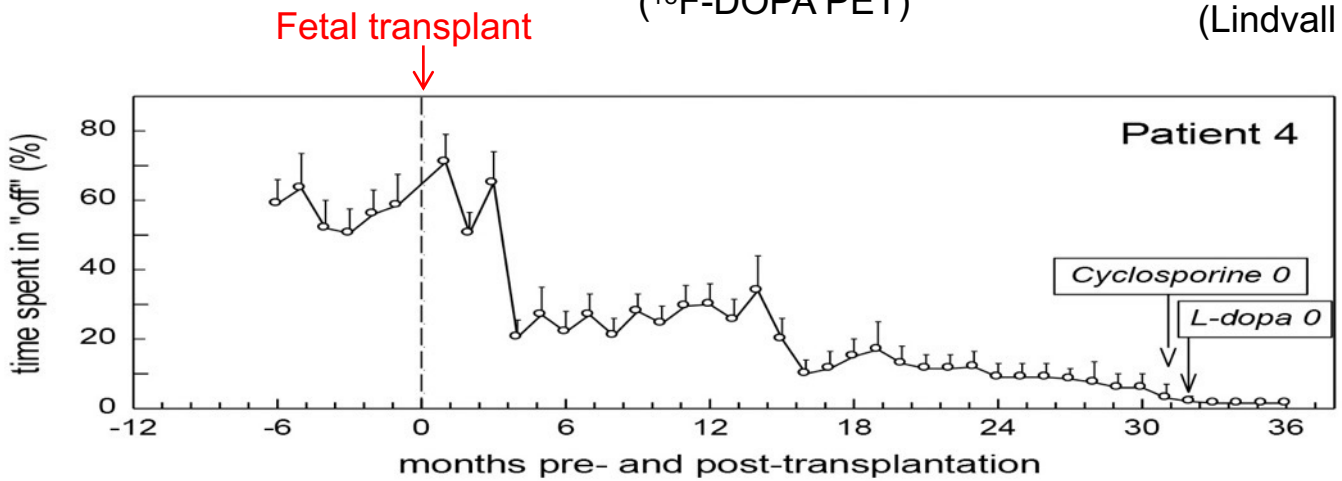
Anders Björklund

Human clinical trials with fetal ventral midbrain tissue

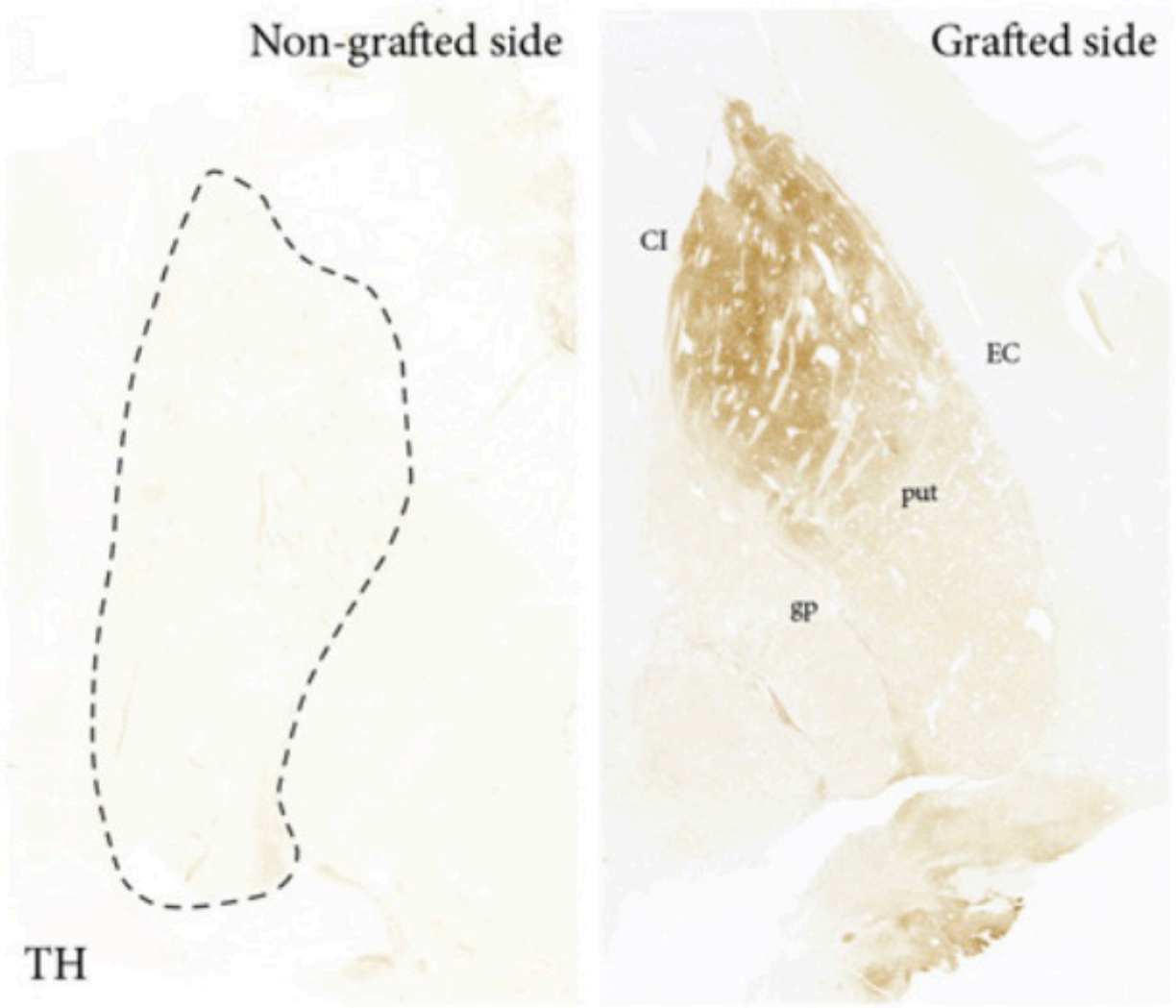


(^{18}F-DOPA PET)

(Lindvall *et al*, 1994)



24 years after fetal transplant



(Li *et al*, 2016, *PNAS*)

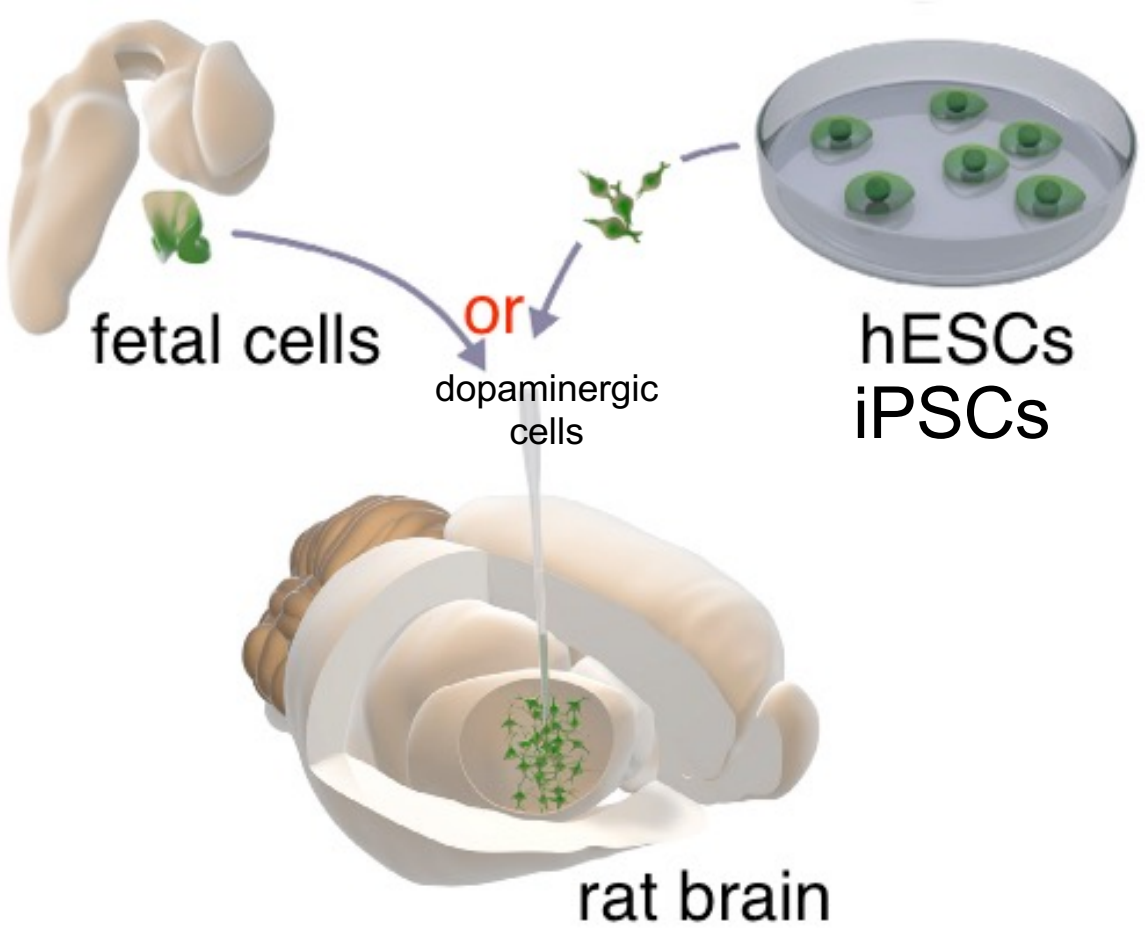
put = putamen
TH = tyrosine hydroxylase

Fetal tissue replaced by stem cell-derived cells

Malin Parmar

Agnete Kirkeby

(hESCs or iPSCs)



Lorenz Studer

(Grealish et al., 2014)

BlueRock THERAPEUTICS

Marcus Yountz *(BlueRock Therapeutics)*

Dopaminergic neuronal precursor cell therapy for Parkinson's disease: background and 1 year results from a Phase 1 study of bemdaneprocel

Marcus Yountz, MD, FAAN · 1st
Vice President, Clinical Development, Neurology at BlueRock Therapeutics

BlueRock Therapeutics

8 Jun 2021

BlueRock Therapeutics Announces First Patient Dosed with DA01 in Phase 1 Study in Patients with Advanced Parkinson's Disease

Tags:

Neurology | Clinical Trials

CAMBRIDGE, Mass., June 8, 2021 — BlueRock Therapeutics, a clinical stage biopharmaceutical company and wholly-owned subsidiary of Bayer AG, announced today the dose administration for the first patient in a Phase 1 (Ph1), open-label study of pluripotent stem cell-derived dopaminergic neurons in patients with advanced Parkinson's disease (PD). The purpose of the Ph1 clinical trial is to evaluate the safety, tolerability, and preliminary efficacy of DA01 in patients with PD.

BlueRock THERAPEUTICS

BlueRock Therapeutics

28 Aug 2023

BlueRock's Phase I study with bemdaneprocel in patients with Parkinson's disease meets primary endpoint

Tags:

Announcement | Neurology

- Investigational cellular therapy, bemdaneprocel (BRT-DA01), was well tolerated with no major safety issues in all 12 participants in low dose and high dose cohorts through one year
- At one-year, exploratory clinical endpoints improved overall, with participants in the high dose cohort showing greater improvement.
- One year assessment of all participants demonstrated feasibility of transplantation, cell survival, and engraftment
- Planning is underway for a Phase II study that is expected to begin enrolling participants in H1 (first half) 2024

Gesine Paul-Visse *(Lund University, Sweden)*

Transplantation of human embryonic-stem cell derived dopaminergic progenitors in Parkinson's disease: first-in-human clinical trial design of STEM-PD

Malin Parmar *(Lund Stem Cell Center, Sweden)*

Developing a stem cell based therapy for Parkinson's disease; the interplay of experimental studies and translational efforts

UK-Sweden trial – February 2023

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LUND UNIVERSITY

UNIVERSITY OF CAMBRIDGE

Malin Parmar

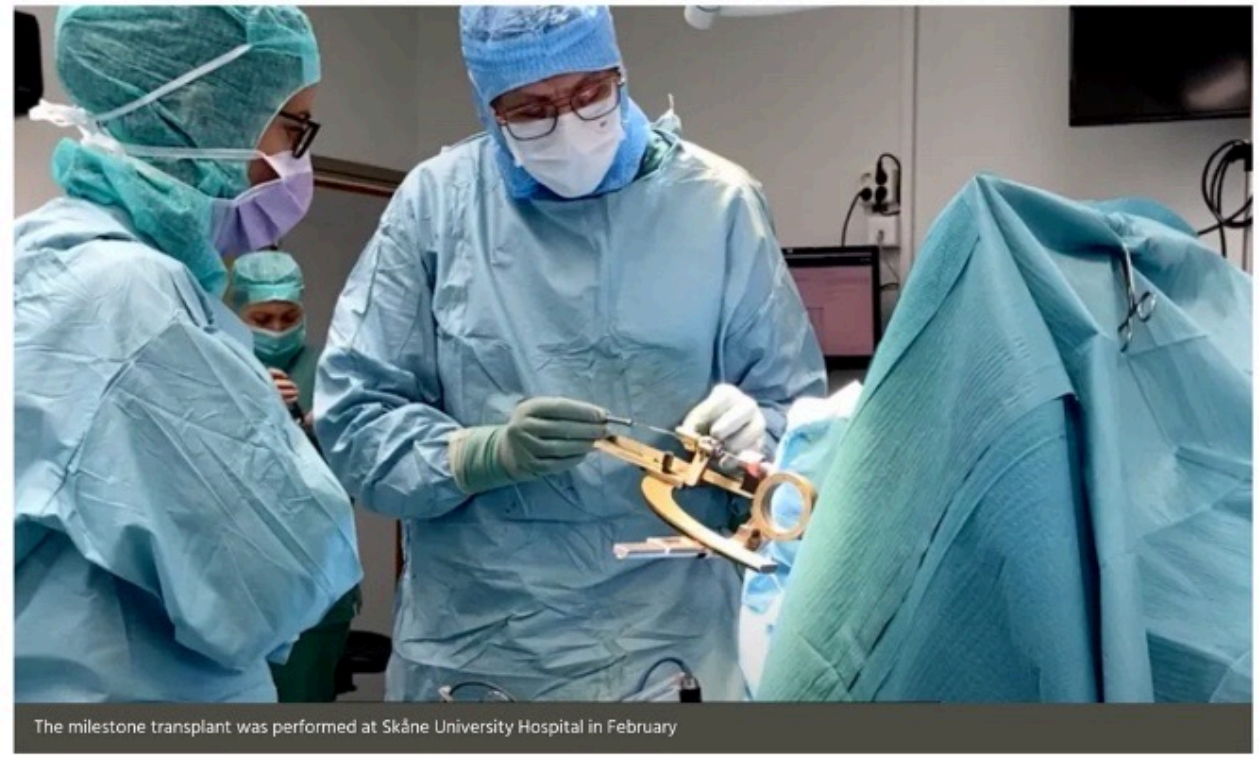
Agnete Kirkeby

Roger Barker

Google “STEM-PD trial”

First patient receives milestone stem cell-based transplant for Parkinson’s Disease

Published 28 February 2023



The milestone transplant was performed at Skåne University Hospital in February

On 13th of February, a transplant of stem cell-derived nerve cells was administered to a person with Parkinson’s at Skåne University Hospital, Sweden. The product has been developed by Lund University and it is now being tested in patients for the first time.

Contact:

Questions regarding the clinical trial:

Gesine Paul-Visse, Senior Consultant

Kristian Kolind *(Novo Nordisk)*

The cell journey - bringing a cell product safely and effectively into patients

Short talk selected from abstract

Merja Voutilainen *(University of Helsinki, Finland)*

CDNF fragment passes the blood brain barrier protecting and restoring dopamine neurons animal models of Parkinson's disease

UNIVERSITY OF HELSINKI

Short talk selected from abstract

Merja Voutilainen *(University of Helsinki, Finland)*

CDNF fragment passes the blood brain barrier protecting and restoring dopamine neurons animal models of Parkinson's disease

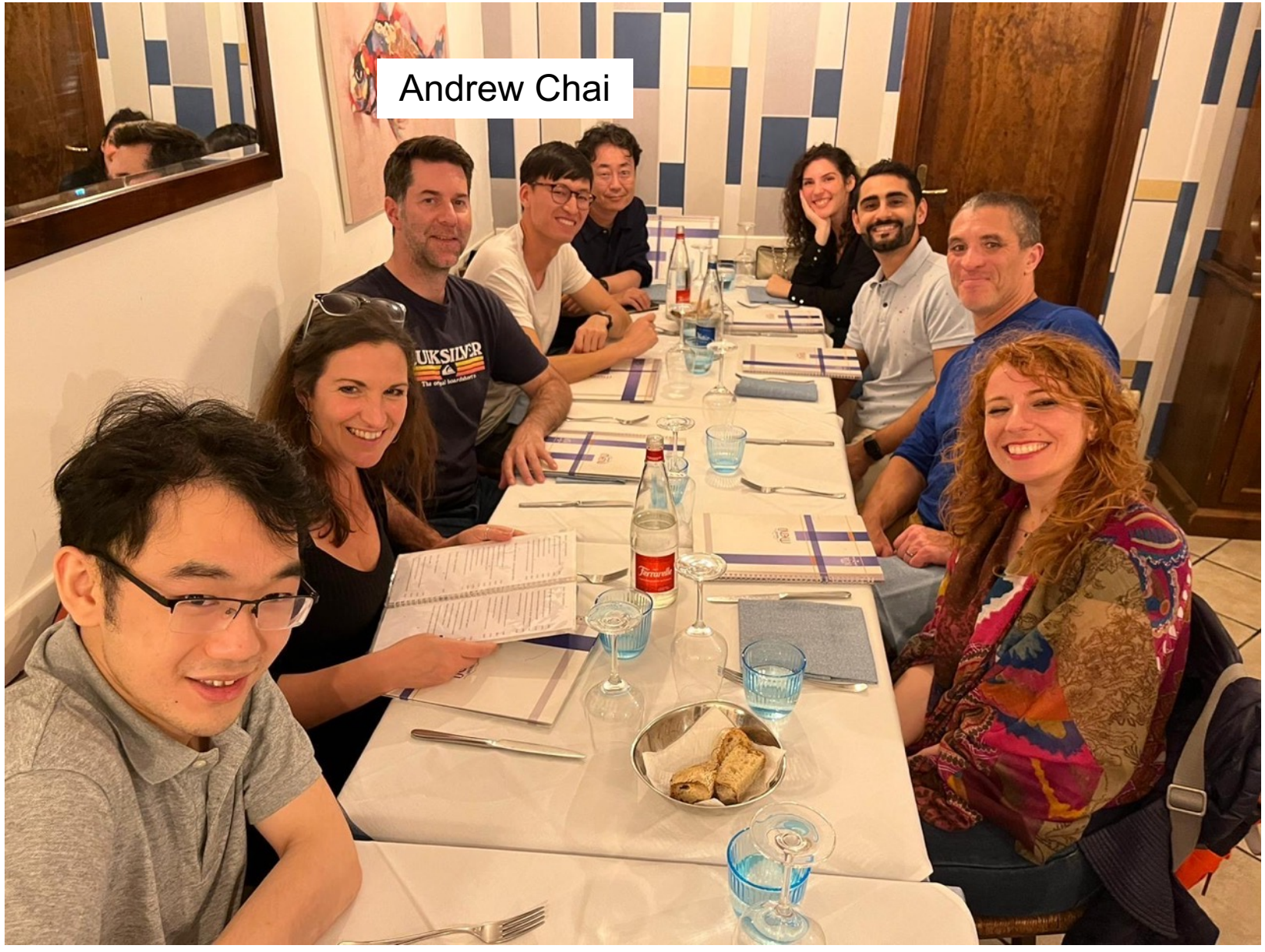
Vol 448|5 July 2007|doi:10.1038/nature05957 nature

LETTERS

Novel neurotrophic factor CDNF protects and rescues midbrain dopamine neurons *in vivo*

Päivi Lindholm[1], Merja H. Voutilainen[2], Juha Laurén[1]†, Johan Peränen[1], Veli-Matti Leppänen[1], Jaan-Olle Andressoo[1], Maria Lindahl[1], Sanna Janhunen[2]†, Nisse Kalkkinen[1], Tõnis Timmusk[1,3], Raimo K. Tuominen[2] & Mart Saarma[1]

Zhijun Wang



Michela Barbato

Anders Björklund

NECTAR President

Romina Aron Badin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Alison Williams & Chiara Zurzolo

Alessandro Fiorenzano

Universita degli Studi di Napoli Frederico II, estd. 1224 AD

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