Europe's definitive event for CNS drug discovery and development

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NeuroDrug

2007

23 - 25 October 2007, The Royal Garden Hotel, London, United Kingdom

Mind the future.

> "NeuroDrug is a forward thinking event that aids participants to examine current hot topics as well as look ahead to the future directions in neurological drug discovery. For this reason, key personnel in academia and industry alike can expect to gain exciting threads of scientific ideas that may lead to research that will ultimately benefit patients with neurological diseases."
>
> **Dr Aaron Chuang,**
> Drug Discovery Stem Cell Coordinator and Manager of Cellular Neurobiology, Neurodegeneration Research Department,
> **GlaxoSmithKline**

> "Without doubt, one of the best I have attended for years, venue, organisation, speakers, content, focus, all unmatched."
>
> **Dr Dale Charlton,**
> Business Development,
> **Bioplan Consulting**
> (Said of our *European Stem Cells and Regenerative Medicine Congress 2007*)

Key speakers include:


Dr Antony Altar
Director of the Biomarkers Consortium
Foundation for the NIH


Dr Trevor Howe
Head of Molecular Informatics
Johnson & Johnson Pharmaceutical Research and Development


Dr Aaron Chuang
Manager of Cellular Neurobiology, Neurodegeneration Research Department
GlaxoSmithKline


Dr Bengt von Manteuffel
Principal Scientist, Molecular Pharmacology Discovery
AstraZeneca


Dr Michael O'Neill
Research Advisor, Neurodegeneration Drug Team
Eli Lilly


Dr Klaus Mendla
Head, CNS Therapeutic Advisory Team, Global Licensing CNS
Boehringer Ingelheim


Dr Nicholas Moore
Associate Director, Behavioural Pharmacology
Lundbeck Research USA Inc.


Dr Lex Schechter
Therapeutic Area Head / Director, Depression and Anxiety Research
Wyeth Research


Dr Henrik Klitgaard
Vice President CNS Research
UCB Pharma SA

CNS disorders: what the future holds for drugs and therapies

- The complete CNS event for science and business strategy
- Dedicated to the development of drugs and therapies for neurological and psychological disorders
- Build new relationships and future partnerships with more hours of networking than any other CNS event
- Case study updates on new targets for neurological, neurodegenerative and psychiatric disorders
- Share industry expertise with leaders from the pharmaceutical and biotech sector

Event sponsor

StemCell Pharma, Inc.

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Mind the future.

Accelerating drug discovery and development for neuro

Day one Wednesday 24 October 2007

KEY ADVANCES IN THERAPEUTICS FOR NEURODEGENERATIVE DISORDERS AND BEYOND

08.00 **Registration and coffee**

08.45 **Chairman's opening remarks**
Dr Eric Parker, Senior Director, Department of Neurobiology, **Schering-Plough Research Institute**

09.00 **Keynote address: neural stem cells as drug targets for neurodegenerative diseases**
- Advancement in the understanding of neurogenesis
- Regulation of neural stem cells
- Pre-clinical models for neurogenesis drug discovery

Dr Aaron Chuang, Drug Discovery Stem Cell Coordinator and Manager of Cellular Neurobiology, Neurodegeneration Research Department, **GlaxoSmithKline**

09.30 **Activating the endogenous stem cells with drugs: a novel approach in clinical trials**
- The potential of stem cell therapies
- Targeting Parkinson's disease, stroke, cognition / mood disorders and orphan CNS indications
- Stem cell based therapies now entering into clinical trials

Dr Anders Haegerstrand, Vice President and Chief Scientific Officer, **NeuroNova**

10.00 **CNS disorders as post traumatic stress diseases (PTSD)**
- High cortisol as a major cause of CNS disorder
- High cortisol as spoiler of stem cell therapy
- Our approach to CNS diseases - amniotic epithelial cells (AEC) plus cortisol modulators

Dr Alfred Sapse, President, Stem Cell Pharma, Inc.

10.30 **Morning coffee**

11.15 **Developing a stem cell therapy for Parkinson's disease**
- Using ReNeuron's c-mycER proprietary technology to develop stable dopaminergic neuronal stem cell line
- Developing new technologies for dopamine neuron delivery to the brain
- Achieving proof of concept

Dr John Sinden, Founder and Chief Scientific Officer, **ReNeuron**

11.45 **Panel session: key successes in stem cell therapies and where we go from here**
- What is the real potential of stem cell therapies?
- A review of results in clinical trials
- Which therapeutic areas show the most potential for success?

Moderator:
Dr Anders Haegerstrand, Vice President and Chief Scientific Officer, **NeuroNova**

Panellists:
Dr John Sinden, Founder and Chief Scientific Officer, **ReNeuron**
Dr Aaron Chuang, Drug Discovery Stem Cell Coordinator and Manager of Cellular Neurobiology, Neurodegeneration Research Department, **GlaxoSmithKline**
Dr Alfred Sapse, President, Stem Cell Pharma, Inc.

12.30 SPEED NETWORKING

13.15 **Lunch**

14.45 **Increasing dopamine synthesis in Parkinson's disease via gene therapy**
- A novel gene-based therapeutic for the treatment of Parkinson's disease
- Using a vector to carry genes encoding the enzymes needed for dopamine synthesis
- Preparation for a Phase I/II human trial in patients with moderate to late-stage Parkinson's disease

Dr Michael McDonald, Chief Medical Officer, **Oxford BioMedica**

15.15 **Gene therapy for neurodegenerative diseases: an AAV genetic delivery mechanism**
- Nonpathogenic adeno-associated virus (AAV) genetic delivery mechanism
- Therapeutic gene unique to each indication
- Applications in Parkinson's disease and epilepsy

John E. Mordock, President and Chief Executive Officer, **Neurologix, Inc.**

15.45 **Attacking beta amyloid accumulation: Alzheimer's antibodies**
- Background: beta-amyloid hypothesis of Alzheimer's disease (AD)
- Active versus passive immunisation approaches
- Studies in transgeneic mouse models of AD
- Clinical trials of anti-Ab immunotherapies in AD patients
- Outlook: future directions in AD immunotherapy

Dr Klaus Mendla, Head, CNS Therapeutic Advisory Team, Global Licensing CNS, **Boehringer Ingelheim**

16.15 **Afternoon tea**

SMALL MOLECULE DRUGS BEING DEVELOPED TO TREAT NEURODEGENERATIVE DISEASE

16.45 **Secretase inhibitors for the treatment of Alzheimer's disease: the cutting edge or cutting it too close?**
- Therapeutic potential: reduction of Ab peptides by BACE and g-secretase inhibitors
- Identification and mitigation of the side effects of g-secretase inhibitors
- Recent insights into the efficacy and side effect potential of BACE inhibitors

Dr Eric Parker, Senior Director, Department of Neurobiology, **Schering-Plough Research Institute**

17.15 **Recent progress and major challenges in the search for disease modifying therapies for Parkinson's disease**
- Mechanisms of cell death
- Neurotoxin models and their use (MPTP, 6-OHDA)
- Pharmacological approaches for neuroprotection and neurorescue
- Newer animal models (proteosome pathway and transgenic model)
- New leads from the study of genetic factors

Dr Michael O'Neill, Research Advisor, Neurodegeneration Drug Team, **Eli Lilly**

17.45 **Neuroprotective targets in excitotoxicity - the postsynaptic density and beyond**
- Excitotoxic neuronal death contributes to many neurological disorders
- Rho as a novel and essential component of the excitotoxic cell death pathway
- Protein-protein interactions specifically involved in neurotoxic signalling

Dr Michael Courtney, Group Leader, Molecular Signalling Laboratory, Department of Neurobiology, **University of Kuopio**

18.15 **Chairman's closing remarks**

18.25 **Networking drinks reception**

CONGRESUL NEURODRUG
Londra, Anglia, 24 -26 Octombrie 2007, Londra, UK

Titlu: „CNS diseases: What the future holds for drugs and therapies" (Maladiile sistemului nervos central: Ce ne aduce viitorul ca medicamente şi tratamente)

La acest congres, doctorul Alfred T. Sapse a fost participant, conferențiar şi membru al echipei de experți, în domeniul "Viitorul celulelor stem".

1. Participants (listă parțială):
   - Dr. Aaron Chuang, GlaxoSmithKline;
   - Dr. Trevor Howe, Johnson & Johnson;
   - Dr. Michael O'Neill, Ely Lilly;
   - Dr. Bengt von Mentzer, Astra-Zeneca;
   - Dr. Les Schechter, Wyeth Research;
   - Dr. Nicholas Moore, Lundberg Research USA
   - Dr. Henrick Klitgardt, UCB Pharma;
   - Dr. Antony Altar, Fundation for the NIH;
   - Dr. Alfred T. Sapse, StemCell Pharma Inc.

2. CONFERINȚA doctorului Sapse, cu titlul "**CNS disorders as post traumatic stress diseases (PTSD)"** (Maladiile stresului sistemului nervos central, cauzate de stresul traumatic), a fost prezentată pe data de 24 octombrie 2007, la ora 10.00 a.m.

La această conferință, Dr. Sapse, a discutat rolul stresului şi al stres hormonului cortizol, în trei maladii neuro-degenerative şi anume Scleroza Multiplă, SLA (Scleroza Laterală Amiotrofică) şi maladia Parkinson.

Dovezile prezentate, au arătat că toate aceste boli, au fost precedate, cu luni şi ani înainte de situații stresante, traumatice, care aplicate soldaților din războiul din Vietnam, a fost numită PTSD (post traumatic stress diseases), datorate războiului, dar care pot interveni şi în viața civilă după episoade foarte stresante de tipul accidentelor de maşină, decese în familie, probleme la locul de muncă, despărțiri conjugale, pierderi financiare şi altele. Datele statistice prezentate, de doctorul Sapse, provenind din arhivele companiei StemCell Pharma Inc., arată că 70-80 % din pacienții afectați de maladiile mai sus precizate, au intervenit după 3-4 luni sau chiar mai mult de un an, după aceste evenimentele. Când nivelul cortizolului total şi liber a fost măsurat la aceşti pacienți, 60%

erau la nivel imuno-supresant, cortizolul distrugând nu numai celulele imune, leucocite, limfocite, dar şi celulele stem embrionare adulte şi progenitoare, în acest fel cortizolul fiind o cauză majoră a maladiilor neurodegenerative (Vezi articolul dr. Alfred T. Sapse: Stess Cortisol Interferon and Stress Diseases, Medical Hypothesis, 13, 31, 1984, Glascow, U. K.). Această noutate, a fost o surpriză pentru reprezentanţii celor mai mari companii farmaceutice, prezente în audienţă, şi o explicaţie de ce studiile clinice cu celule stem au eşuat în trecut, datorită ignorării efectelor imuno-distructive a cortizolului.

Programul Celule Stem al companiei StemCell Pharma Inc. urmăreşte două obiective: 1) **de reparare** a distrugerilor cauzate de cortizol prin utilizarea de celule stem şi 2) **de tratare a cauzei** acestor leziuni prin utilizarea de medicamente anticortizol, sau modulatoare de cortizol. Intrând în detalii asupra tratamentului cu celule stem, doctorul Sapse a menţionat rolul telomerazei, o enzimă care controlează numărul de multiplicări a celulelor stem. La acest subiect, a menţionat metodele StemCell Pharma de creştere a lungimii telomerazei, inclusiv ale numărului telomerilor, care rezultă într-o prelungire a vieţii celulelor stem.

De exemplu, celulele stem întrebuinţate de StemCell Pharma Inc, provin din membrana amniotică a placentei. Aceste celule care se comportă ca celule embrionare, în sensul că ele pot să repare şi chiar să reconstruiască toate tipurile de celule, ţesuturi sau organe, au totuşi o deficienţă majoră şi anume, că ele se pot multiplica numai de 20 de ori, timp prea scurt ca să exercite calităţile lor reparatoare. Prin tehnica doctorului Sapse, lungimea telomerazei (şi numărul de telomeri), creşte de 3 ori, adică poate merge până la 60 de multiplicări, care asigură timpul suficient (4-6 luni) pentru ca să-şi termine misiunea lor de reparare sau reconstruire.

În concluzie, doctorul Sapse, a propus acceptarea Sclerozei Multiple, SLA şi a maladiei Parkinson, ca maladii post traumatice (PTSD post traumatic stress diseases), clasificare care ar antrena în mod automat măsurarea cortizolului şi introducerea combinaţiei anticortizol, cu tratamentul cu celule stem.

Această propunere va fi înaintată la NIH (National Institute of Health), pentru evaluare. Doctorul Sapse este un "NIH, Special Research Fellow", al acestei organizaţii.

3. Doctorul Sapse a fost ales în comisia de experţi în celule stem (panel team).

Comisia este alcătuită din următorii:

Panel Team:
Dr Erik Miljan, Directorul Stem Cell Discovery, **ReNeuron**
Dr Aaron Chuang, Drug Discovery Stem Cell, Coordonator şi Manager la Cellular Neurobiology, Neurodegeneration Research Department, **GlaxoSmithKline**
Dr Alfred Sapse, Preşedinte al **Stem Cell Pharma, Inc.**

Pe data de 24 octombrie 2007, la ora 11.45, comisia de experţi s-a întrunit pentru a discuta subiectul "Cheia succesului în terapia cu celule stem şi unde ne îndreptăm acum?" („Key succes in Stem Cell therapy, and where do we go from here?").

Cu această ocazie, biografiile doctorului Chuang şi a doctorului Sapse au fost prezentate, totodată au fost făcute fotografii de către agenţiile de presă.

Dr Aaron Chuang
Drug discovery stem cell coordinator and Manager of Cellular Neurobiology,
Neurodegeneration Research Department
GlaxoSmithKline

Following PhD training in Neuroendocrinology at St. Thomas's Hospital Medical School in London, Aaron studied the molecular regulation of gene expression in the pituitary gland at INSERM in Paris. This led to research on G Protein-Coupled Receptor Kinases at the Mario Negri Institute in Italy, where his work shed light on the disease implications of these kinases. Subsequently Aaron joined GlaxoWellcome in 1996 to apply this expertise towards drug discovery in respiratory diseases. After the formation of GlaxoSmithKline in 2001, Aaron moved into drug discovery for CNS diseases, and has led programmes in early and late preclinical stages for Alzheimer's Disease. During the same period he initiated a stem cell research programme for neurodegenerative diseases. Aaron is also involved in international business development opportunities, and serves on the editorial board for Regenerative Medicine.

Dr Alfred Sapse
President
Stem Cell Pharma, Inc.

Education:

MD	School of Medicine, University of Bucharest, Romania	1952
	Residency in Ophthalmology	
	University Eye Clinic, Bucharest, Romania	1959-1960
	University Eye Clinic, Geneva, Switzerland	1963-1964
	Postdoctoral Microbiology	
	University of California at Los Angeles	1969-1972

Professional Background:

Assistant Professor, University Eye Clinic	
Professor A. Francheschetti, Geneva, Switzerland	1964-1965
Research Associate, Eye Research	
Cedars-Sinai Medical Center, Los Angeles, CA	1965-1967
Director, Laboratory of Ophthalmic Immunology	
Cedars-Sinai Medical Center, Los Angeles, CA	1968-1971
Assistant Professor IV, Department of Bacteriology	
University of California, Los Angeles, UCLA	1968-1971
NIH Special Research Fellow (1968)	

Society Membership:
American Medical Association (AMA)
New York Academy of Sciences
French Society of Ophthalmology
American Association of Ophthalmology
American Geriatric Society
Pan American Medical Association

Vice President of Research and Development	
Spectrum Pharmaceutical Corporation	August 1989 - March 1992
CEO, President and Director of Research	
Steroidogenesis Inhibitors, Inc.	
(Presently Samaritan Pharmaceuticals LIV – AMEX)	September 1992 – June 2000

<u>Panel Team</u>: Dr. Aaron Chuang, GlaxoSmithKline şi Dr. Alfred Sapse, StemCell Pharma, Inc.

În timpul discuțiilor, doctorul Chuang a surprins audiența când a relatat că el cunoaşte activitatea doctorului Sapse în domeniul cortizol şi celule stem, adăugând totodată, că GlaxoSmithKline, studiază în prezent mijloace de tratament al maladiei Alzhaimer unde cortizolul joacă un rol important în inițierea maladiei, urmată de distrugerea celulelor receptoare de cortizol din hippocampus (centrul memoriei din creier) şi activarea secretazei, o enzimă neuro-distructivă.

Doctor Sapse a menționat că are două patente cu subiectul tratamentul Alzhaimer, emise de către Departamentul de Patente ale USA.

Congresul NEURODRUG 2007 a fost un succes important pentru doctorul Sapse. În prezent discuții sunt în curs cu două companii farmaceutice interesate de programul StemCell Pharma Inc. Doctorul Sapse a fost invitat să participe la "European Stem Cell Regenerative Medicine Congress", care va avea loc în Londra între 13-15 mai 2008, unde va prezenta o conferință cu titlul: „Transsclera, non-invasive stem cell therapy of retina diseases". **Target diseases:** RP, LCA, Usher's syndrome, Stargardt and others. **Target area:** Retina photoreceptors, cones and rodes area. **Target results:** Differentation of stem cells into photoreceptor cells.