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## Medienmappe Annemarie Opprecht Parkinson Award 2012

9. November 2012

### 5. Annemarie Opprecht Parkinson Award 2012

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## 5. Annemarie Opprecht Parkinson Award 2012

### **Schweizerische Annemarie Opprecht-Stiftung verleiht zum 5. Mal ihren mit 100 000 Franken dotierten Parkinson-Forschungspreis. Preisträger ist der gebürtige Schweizer Prof. Lorenz Studer**

**Basel – Die Schweizerische Annemarie Opprecht-Stiftung und Parkinson Schweiz haben heute in Basel zum fünften Mal den Annemarie Opprecht Parkinson Award für hervorragende Forschungsarbeiten auf dem Gebiet der Parkinsonkrankheit verliehen. Der mit 100 000 Franken dotierte internationale Forschungspreis ging an den in Solothurn geborenen und heute in New York wohnhaften Neurowissenschaftler Professor Lorenz Studer.**

**Professor Lorenz Studer wurde für seine Arbeit «Dopamine neurons derived from human ES cells efficiently engraft in animal models of Parkinson's disease», Nature 2011, ausgezeichnet.**

Bereits zum fünften Mal verlieh die Schweizerische Annemarie Opprecht-Stiftung dieses Jahr ihren Forschungspreis für hervorragende wissenschaftliche Arbeiten auf dem Gebiet der Parkinsonkrankheit. Die 1998 von der selbst an Parkinson erkrankten Philantropin Annemarie Opprecht gegründete und finanzierte Stiftung hat das Ziel, die medizinische Parkinsonforschung auf internationaler Ebene zu fördern, wobei sie eng mit Parkinson Schweiz kooperiert.

Der mit 100 000 Franken dotierte Annemarie Opprecht Parkinson Award zählt weltweit zu den bedeutendsten neurologischen Forschungspreisen. 1999 wurde er erstmals an die Neurologen Anthony Shapira, London, und Pierre Pollak, Grenoble, vergeben. 2002 wurde der Spanier Jose Obeso ausgezeichnet, 2005 erhielten die beiden US-amerikanischen Forscher Zbigniew K. Wszolek, Jacksonville (FL), und Stanley Fahn, New York (NY), den Preis zu gleichen Teilen. 2008 hiessen die Preisträger John A. Hardy, London, und Prof. Andrew B. Singleton, USA.

2012 gab es eine Premiere zu feiern: Mit Professor Lorenz Studer wurde erstmals ein Schweizer mit dem Annemarie Opprecht Parkinson Award ausgezeichnet. Der weltweit renommierte Forscher studierte Medizin an den Universitäten Fribourg und Bern und doktorierte 1994 an der Universität Bern in Neurowissenschaften.

Im Anschluss absolvierte er ein postgraduate Fellowship zum Thema Neurotransplantation an der Neurochirurgischen Klinik des Universitätsspitals am Berner Inselspital, ehe er im Jahr 1996 ein Research Fellowship am renommierten National Institutes of Health (NIH) in Bethesda, USA, mit Forschungsschwerpunkt Stammzellbiologie antrat. 2000 folgte der Wechsel nach New York in die Abteilung für Entwicklungsbiologie und Neurochirurgie des bekannten Memorial Sloan-Kettering Cancer Center (MSKCC). Zugleich wurde Lorenz Studer Professor für Developmental Neurobiology an der Cornell Medical School, New York. Seit 2010 ist er Direktor des Zentrums für Stammzellbiologie (Center for Stem Cell Biology, CSCB) am MSKCC in New York.

Die Forschungsarbeiten von Lorenz Studer konzentrieren sich auf die Herstellung spezifischer Nervenzellen, welche die Dopamin produzierenden Neuronen, die bei Parkinsonpatienten degenerieren, ersetzen könnten. Er und seine Forschungsgruppe entwickelten neuartige Methoden, die es erlauben, menschliche pluripotente Stammzellen (humane PS-Zellen) in dopaminerge Neuronen umzuwandeln. Insbesondere konnten Lorenz Studer und sein Team belegen, dass diese Zellen sowohl in vitro (im Reagenzglas) als auch in vivo (im Tierversuch mit Maus-, Ratten- und Affenmodellen der Parkinsonkrankheit) überleben und die Funktion der bei Parkinson degenerierenden Zellen übernehmen können. Da genau die Degeneration dieser Zellen den Grundprozess der Parkinsonkrankheit darstellt, repräsentiert die Transplantation von aus PS-Zellen hergestellten dopaminergen Neuronen die derzeit einzige potenziell kausale Therapiemöglichkeit bei Parkinson. Da es die von Lorenz Studer entwickelte Methode erlaubt, Dopamin produzierende Zellen aus den pluripotenten Stammzellen jedes einzelnen Menschen herzustellen, könnte für eine solche Therapie körpereigenes Gewebe der betroffenen Patienten verwendet werden - womit auf aus Embryonen gewonnene Stammzellen verzichtet werden kann.

Diese Entwicklung markiert einen grossen Fortschritt in der Entwicklung möglicher künftiger Zellersatztherapien bei der Parkinsonkrankheit. Entsprechende Ansätze auf Basis der Transplantation embryonaler (aus Föten gewonnener) mesencephaler Zellen wurden vor mehreren Jahren nach Misserfolgen quasi «begraben».

Die Herstellung dopaminergere Zellen aus körpereigenen pluripotenten Stammzellen ermöglicht des Weiteren ganz neue Ansätze in der Erforschung medikamentöser Therapieformen bei der Parkinsonkrankheit.

Das Preiskomitee der Annemarie Opprecht Stiftung und der Forschungsausschuss von Parkinson Schweiz gratulieren Prof. Lorenz Studer und seiner Forschungsgruppe zu diesen wertvollen Errungenschaften im Kampf gegen die Parkinsonkrankheit.

**Für persönliche Auskünfte kontaktieren Sie bitte:**

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## 5. Annemarie Opprecht Parkinson Award 2012

### Swiss Annemarie Opprecht-Foundation awards Parkinson research

**Basel – The Swiss Annemarie Opprecht-Foundation and the Swiss Parkinson's Disease Society (Parkinson Schweiz) have awarded outstanding work in Parkinson's disease research on Friday, November 9th 2012 in Basel, Switzerland.**

The research award in the amount of 100'000 Swiss francs (appr. 82'000 Euro) was given to the Researcher **Lorenz Studer** (MD, Professor Developmental Neurobiology, Cornell Medical School, New York and Director, SKI Center for Stem Cell Biology, Sloan-Kettering Institute for Cancer Research, New York, USA).

The price was given for the fifth time since the foundation's establishment in 1998. Its aim is strictly to promote scientific research on Parkinson's disease.

Lorenz Studer is the Director of the Sloan-Kettering Center for Stem Cell Biology. He is a Member of the Developmental Biology Program and the Department of Neurosurgery at MSKCC and a Professor in Neuroscience at Weill-Cornell Medical School in New York. Early contributions of his lab include the in vitro derivation of midbrain dopamine neurons from ES, nuclear transfer ES cells and parthenogenetic stem cells. His laboratory was also first to demonstrate «therapeutic cloning» in a mouse model of a CNS disorder, and he has pioneered studies on the directed differentiation, high-throughput screening and genetic modification of human ES cells. His most recent work increasingly focuses on the translational application of human pluripotent stem cells in disease modeling, drug discovery and cell therapy.

Lorenz Studer is honoured for his study «Dopamine neurons derived from human ES cells efficiently engraft in animal models of Parkinson's disease», published in Nature 2011.

#### **For further informations:**

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## 5. Annemarie Opprecht Parkinson Award 2012

### Sieben persönliche Fragen an den Preisträger Lorenz Studer

#### Was bedeutet Ihnen die Auszeichnung mit dem Annemarie Opprecht Parkinson Award 2012?

Natürlich freue ich mich sehr, dass ich – bei der fünften Verleihung dieses internationalen Forschungspreises – als erster Schweizer überhaupt diesen Preis erhalten habe. Es ist eine grosse Ehre für mich als Schweizer – und als Stammzellforscher. Die Stammzellforschung war oft nicht sehr «Main-stream» und die bisherigen Preisträger für diesen Award waren die renommiertesten Parkinsonforscher auf dem Gebiet der traditionellen Forschungsrichtungen.

Mit der Auszeichnung meiner Person wird nun auch die Rolle der Stammzellforschung im Bereich der Parkinsonerkrankung geehrt. Da auch der Nobel-Preis für Physiologie und Medizin dieses Jahr an Shinya Yamanaka ging, einen Forscher aus dem Bereich der Stammzellforschung, wird deutlich, dass unsere Forschungsrichtung grosse Anerkennung in der Wissenschaft und in der Medizin erfährt. Wir hoffen natürlich, dass unsere Forschung schlussendlich auch grosse medizinische Durchbrüche bringen wird.

#### Sie verwenden für Ihre Forschungen Zellen aus menschlichen Embryonen. Haben Sie keine ethischen Bedenken?

Ich komme aus einem katholisch geprägten Umfeld und habe mich entsprechend auch mit den ethischen Fragen meiner Arbeit auseinandergesetzt. Ich sehe persönlich keine Bedenken, solange die Forschung unter korrekten ethischen Bedingungen durchgeführt wird. Diese ethischen Bedingungen eingehend zu diskutieren, würde lange dauern. Daher nur so viel: Die embryonalen Stammzellen, welche wir für unsere Studien verwenden, wurden vor mehr als 10 Jahren hergestellt, und zwar aus «Material», welches ohnehin als «medizinischer Abfall» entsorgt worden wäre.

Zudem funktioniert unsere Methode auch mit sogenannten iPSC-Zellen (indizierte pluripotente Stammzellen), die sich zwar wie embryonale Stammzellen verhalten, aber via Reprogrammierung von Hautzellen generiert wurden (siehe Nobelpreis 2012 für Shinya Yamanaka). Diese werden heute als ethisch unbedenkliche Alternative zu embryonalen Stammzellen verwendet.

#### Wird Parkinson dank Ihrer Forschung schon bald heilbar sein?

Wir denken, dass sich Zellersatztherapien auf der Basis von Stammzellen zu einer interessanten neuen Therapie für die Behandlung von Parkinson entwickeln werden. Jedoch wird dies sehr wahrscheinlich nicht eine komplette Heilung bringen, aber doch hoffentlich eine gute Alternative zu den herkömmlichen Therapien. Im Moment befinden wir uns im Stadium, in dem wir die klinische Anwendung planen können und wir hoffen, dass die ersten Patienten in etwa 4 Jahren behandelt werden können. Zusätzlich kann unsere Methode auch dazu benutzt werden, um neue Medikamente für die Anti-Parkinson-Therapie zu entwickeln (patient-specific disease modeling and drug discovery).

**Sie sind 1996, nach dem Studium in Bern, in die USA gegangen, wo Sie bis heute leben. Warum haben Sie damals die Schweiz verlassen und welche Beziehung haben Sie heute zu Ihrer Schweizer Heimat?**

Ich bin in Solothurn geboren und wuchs in Hagendorf (SO) auf, besuchte dort auch die Primarschule und dann das Gymnasium an der Kanti Olten. Danach studierte ich Medizin in Fribourg und Bern, wo ich auch meine ersten Forschungsarbeiten durchführte (MD und zusätzliche Doktorarbeit in Neurowissenschaften).

Wie viele andere junge Forscher ging ich in die USA, um mich dort weiterzubilden (Postdoctoral Fellowship mit Stipendium der Swiss NSF). Nach der Weiterbildung, so war es geplant, wollte ich wieder in die Schweiz zurückkehren. Doch dazu kam es nicht. Einerseits, weil die Stammzellforschung in den USA viel fortgeschrittener war als in der Schweiz, andererseits gab es aber auch private Gründe. Ich habe in den USA meine Lebenspartnerin kennengelernt und mit ihr eine Familie gegründet.

Obwohl ich nun schon mehr als 15 Jahre in den USA lebe, habe ich aber doch noch enge und auch regelmässige Kontakte mit der Schweiz. Denn meine Familie (Eltern, Geschwister, Enkel etc.) leben ja alle hier in der Schweiz.

**Sie gingen der Forschung halber in die USA. Ist dort das Umfeld für Forscher besser als in der Schweiz?**

Das kann man nicht so generalisieren. Die Schweiz hat bekanntermassen ein sehr gutes Umfeld für viele Forschungsrichtungen. In der Stammzellforschung allerdings sind die USA wesentlich weiter. In New York ist das Umfeld diesbezüglich speziell gut, da hier gleich drei grosse Institute (Memorial Sloan-Kettering, Cornell University und Rockefeller University) im gleichen Quartier angesiedelt sind, also «across the street», wie wir hier sagen. Zum Beispiel haben wir hier die «Tri-institutional stem cell initiative», die private Gelder für die Stammzellforschung an diesen drei Instituten zur Verfügung stellt. Entsprechend findet sich dort auch eine sehr grosse Zahl von Spitzenforschern für Kooperationsprojekte.

**Apropos New York: Wurde Ihre Arbeit durch den Sturm beeinträchtigt?**

Glücklicherweise hatten wir keine grossen Schäden zu beklagen, weder bei uns zuhause, noch im Labor. Doch einige unserer Nachbarinstitute, vor allem an der New York University hatten sehr grosse Schäden. Sie haben fast alle Tierkolonien und Reagenzien im Sturm verloren.

**Erzählen Sie uns bitte noch ein paar persönliche Fakten. Wo leben Sie? Haben Sie Kinder? Sind Sie schon US-Bürger – und falls ja: Welcher Präsidentschaftskandidat hat Ihre Stimme erhalten?**

Ich bin verheiratet und wir haben zwei Kinder, Alexander (11) und Yara-Maria (9). Wir leben in der Upper East Side of Manhattan und meine Frau arbeitet als Neurochirurgin und Forscherin am Memorial Sloan-Kettering Institute. Sie ist übrigens auch eine Ko-Autorin der Publikation, für welche ich – also wir – nun mit dem Annemarie Opprecht Parkinson Award ausgezeichnet wurden.

Ich bin bis heute Schweizer Bürger, besitze «nur» eine Green Card (permanent resident). Daher kann ich in den USA nicht wählen. Aber ich stimme regelmässig via Internet in der Schweiz ab. Bei den Präsidentschaftswahlen in den USA hätte klar Obama meine Stimme erhalten. Ein direkter Grund in Bezug zu meiner Forschung ist die Tatsache, dass Mitt Romney gegen die Stammzellforschung ist!

## 5. Annemarie Opprecht Parkinson Award 2012

### Die Annemarie-Opprecht-Stiftung

Am 20. März 1998 hat die Philanthropin Annemarie Opprecht-Grollmund (\* 11. 2. 1925) aus Bergdietikon AG eine gemeinnützigen Stiftung mit einem Vermögen von einer Million Schweizer Franken eingerichtet.

Die Stiftung mit Sitz in Aarau fördert die internationale, medizinische Forschung in allen Bereichen der Parkinsonkrankheit. Dazu verleiht sie im Drei-Jahres-Rhythmus den internationalen Annemarie Opprecht Parkinson Award für Forschungsarbeiten, die gewichtige Ergebnisse zur Erforschung der Parkinsonkrankheit in allen Bereichen (beispielsweise Grundlagenforschung, Epidemiologien, Therapien, Genetik) beinhalten.

#### **Preiskomitee**

Das Preiskomitee der Annemarie Opprecht-Stiftung setzt sich derzeit aus acht internationalen Fachautoritäten im Bereich der Parkinsonkrankheit zusammen. Präsident des Preiskomitees ist Prof. Dr. med. Matthias Sturzenegger, Leitender Arzt des Neurologischen Universitätsspitals am Inselspital Bern sowie Mitglied des Vorstandes und des Fachlichen Beirates von Parkinson Schweiz. Mit den Professoren José A. Obeso, Anthony H. V. Schapira und Zbigniew K. Wszolek nehmen auch drei ehemalige Preisträger des Annemarie Opprecht Parkinson Award Einsitz im Preiskomitee.

- Prof. Mathias Sturzenegger, Schweiz (Präsident)
- Prof. Pierre Burkhard, Schweiz
- Prof. Günther Deuschl, Deutschland
- Prof. Joseph-André Ghika, Schweiz
- Prof. Mark Hallett, USA
- Prof. José A. Obeso, Spanien
- Prof. Anthony H.V. Schapira, England
- Prof. Zbigniew K. Wszolek, USA

#### **Preissumme**

Für die Preissumme sind primär die Erträge des Stiftungskapitals zu verwenden, wobei ein Betrag je Preisverleihung von CHF 100'000. – angestrebt wird.

#### **Kandidatur**

Kandidaten für den Annemarie Opprecht Parkinson Award können sowohl dem universitären als auch dem kommerziellen Forschungsbereich entstammen. Der Forschungspreis wird einmal in einer internationalen neurologischen Fachzeitschrift ausgeschrieben. Jedes Mitglied des Preiskomitees kann zusätzlich Kandidaten einladen, ihre Arbeit einzureichen. Die Arbeiten der Kandidaten müssen publiziert oder zur Publikation bereits akzeptiert sein. Sie sind in englischer Sprache abzufassen.

Preisempfänger können Einzelpersonen oder ein Team sein. Der Preisträger präsentiert seine Forschungsergebnisse anlässlich der Preisverleihung im Rahmen eines Referates.

**Die bisherigen Preisträger:****1999**

Prof. A.H.V. Schapira, University College London, London, Great Britain

*Award-winning publication: «Mitochondrial DNA Transmission of the Mitochondrial Defect in Parkinson's Disease», Ann Neurology 1998; 44:177-186.*

Prof. Pierre Pollak, Joseph Fourier University, Grenoble, France

*Award-winning publication: «Electrical Stimulation of the Subthalamic Nucleus in Advanced Parkinson's Disease», New Engl J Med 1998; 339: 1105-1111.***2002**

Prof. José A. Obeso, Universidad de Navarra, Pamplona, Espania

*Award-winning publication: «The subthalamic nucleus in Parkinson's Disease: Somatotopic organization and physiological characteristics», Brain 2001; 124: 1777-1790.***2005**

Prof. Stanley Fahn, Neurological Institute, Columbia University Medical Center, New York, USA.

*Award winning publication: Parkinson Study Group, «Levodopa and the progression of Parkinson's disease», N Engl J Med. 2004 Dec 9; 351(24): 2498 - 2508.*

Prof. Zbigniew K. Wszolek, Department of Neurology, Mayo Clinic Jacksonville, Jacksonville, USA.

*Award winning publication: «Mutations in LRRK2 cause autosomal-dominant parkinsonism with pleomorphic pathology», Neuron 2004; 444: 601-607.***2008**

Professor John Hardy, Institute of Neurology, University College, Queen Square, London, and Prof. Andrew B. Singleton, Chief of the Molecular Genetics Section and Acting Chief of the Laboratory of Neurogenetics, National Institute on Aging, National Institutes of Health, Bethesda, Maryland, USA

*Award winning publication: «Genome-wide genotyping in Parkinson's disease and neurologically normal controls: first stage analysis and public release of data», Lancet Neurol 2006; 5: 911-16.***2012**

Professor Lorenz Studer, Professor Developmental Neurobiology, Cornell Medical School, New York and Director, SKI Center for Stem Cell Biology, Sloan-Kettering Institute for Cancer Research, New York, USA

*Award winning publication: Dopamine neurons derived from human ES cells efficiently engraft in animal models of Parkinson's disease. Nature 2011.***Kontakt zur Annemarie Opprecht-Stiftung:****Vizepräsident des Stiftungsrates:** Prof. Dr. rer. pol. Peter Gurtner, Schweiz**Sekretariat:**Parkinson Schweiz, Gewerbestrasse 12a, Postfach 123, CH-8132 Egg, Tel. 043 277 20 77, Fax 043 277 20 78, E-Mail: [aofoundation@parkinson.ch](mailto:aofoundation@parkinson.ch)Websites: [www.opprecht-foundation.com](http://www.opprecht-foundation.com) und [www.parkinson.ch](http://www.parkinson.ch)Bureau romand Chemin des Charmettes 4, 1003 Lausanne, tél./fax 021 729 99 20, [info.romandie@parkinson.ch](mailto:info.romandie@parkinson.ch)  
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## 5. Annemarie Opprecht Parkinson Award 2012

### CV Professor Lorenz Studer

Memorial Sloan-Kettering Cancer Center  
New York, New York

Lorenz Studer ist in der Schweiz geboren, graduierte 1991 in Medizin und erhielt 1994 seinen Doktorgrad in Neurowissenschaften von der Universität Bern. Während er dort arbeitete, initiierte er Studien mit Christian Spenger, welche in die ersten klinischen Tests fötaler Zelltransplantation für die Parkinsonkrankheit in der Schweiz mündeten. Danach führte Studer seinen Forschungsarbeiten an den National Institutes of Health (NIH) in Bethesda, Maryland, fort, wo er in den Laboratorien von Ron McKay arbeitete. Am NIH leistete er Pionierarbeit für die Herstellung dopaminergischer neuronaler Zellen und war 1998 der erste, der zeigen konnte, dass die Transplantation solcher in Kulturen hergestellten Dopamin produzierenden Zellen die Symptome im Tiermodell bei Parkinsonmäusen verbessern kann.

Im Jahr 2000 verlagerte er seine Arbeit nach New York City wo er sein Forschungsprogramm am Memorial Sloan-Kettering Cancer Center (MSKCC) startete. Frühe Beiträge seines Labors schliessen die in-vitro-Herstellung mesencephaler dopaminergischer Neuronen aus embryonalen Stammzellen (ES), von Nuclear-Transfer-ES-Zellen und von parthenogenetischen Stammzellen ein.

Sein Labor war überdies das erste, das «therapeutisches Klonen» in einem Mausmodell für ZNS-Krankheiten demonstrieren konnte. Überdies leistete Lorenz Studer Pionierarbeit für Studien zur gezielten Differenzierung, zum High Throughput Screening und zur genetischen Modifikation menschlicher embryonaler Stammzellen.

Seine jüngsten Arbeiten fokussieren auf die translationelle Anwendung menschlicher pluripotenter Stammzellen (PS-Zellen) als Krankheitsmodelle für die Entwicklung neuer Medikamente und für die Zellersatztherapie. Er erhielt zahlreiche Preise für seine Arbeit, beispielsweise den Boyer Young Investigator Award und, am 9. November 2012 in Basel, den Annemarie Opprecht Parkinson Award 2012.

Lorenz Studer ist Direktor des Sloan-Kettering-Zentrums für Stammzellbiologie, Mitglied des Biologischen Entwicklungsprogrammes und der Neurochirurgie des MSKCC sowie Professor für Neurowissenschaften an der Weill-Cornell Medical School in New York.

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## 5. Annemarie Opprecht Parkinson Award 2012

### CV Lorenz Studer MD

Memorial Sloan-Kettering Cancer Center  
New York, New York

A native of Switzerland, Lorenz Studer graduated from medical school in 1991 and received his doctoral degree in neuroscience at the University of Bern in 1994. While there, he initiated studies with Christian Spenger, leading to the first clinical trial of fetal tissue transplantation for Parkinson's disease in Switzerland. Studer next pursued his research interests at the National Institutes of Health (NIH) in Bethesda, Maryland, where he worked in the laboratory of Ron McKay. At the NIH he pioneered the derivation of dopamine cells from dividing precursor cells. In 1998, he was first to demonstrate that the transplantation of dopamine cells generated in culture improve behavioral symptoms in Parkinsonian rats.

In 2000, he moved to New York City where he started his research program at the Memorial Sloan-Kettering Cancer Center (MSKCC). Early contributions of his lab include the in vitro derivation of midbrain dopamine neurons from ES, nuclear transfer ES cells and parthenogenetic stem cells. His laboratory was also first to demonstrate "therapeutic cloning" in a mouse model of a CNS disorder, and he has pioneered studies on the directed differentiation, high-throughput screening and genetic modification of human ES cells. His most recent work increasingly focuses on the translational application of human pluripotent stem cells in disease modeling, drug discovery and cell therapy. He received numerous awards for his work including the Boyer Young Investigator award and, most recently, the Annemarie Opprecht Award.

Studer is the Director of the Sloan-Kettering Center for Stem Cell Biology. He is a Member of the Developmental Biology Program and the Department of Neurosurgery at MSKCC and a Professor in Neuroscience at Weill-Cornell Medical School in New York.

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## 5. Annemarie Opprecht Parkinson Award 2012

### Lorenz Studer, Tätigkeiten und Profil

#### Affiliations:

- Developmental Biology Program, Neurosurgery, Center for Cell Engineering, Center for Stem Cell Biology

#### Research topics

- Cell Biology; Cell Signaling; Gene Regulation; Neuroscience; Stem Cell Biology

Our laboratory aims at exploiting recent advances in stem cell biology to develop radically new therapies for degenerative disease and cancer. We work with both embryonic and adult stem cell types in the lab. However, the main current focus is on the biology and use of human embryonic stem cells. Embryonic stem cells may provide a truly unlimited source for deriving therapeutically relevant cell types. In the mouse, studies have demonstrated that embryonic stem cells can give rise to virtually any cell type present in the adult organism.

A major effort of the lab is devoted to harnessing and manipulating the differentiation potential of embryonic stem cells. The efficient generation of specific brain cell populations in vitro, such as dopamine, GABA, motor neurons, or myelinating oligodendrocytes, can serve as a potential cellular source for brain repair in Parkinson's Disease, Huntington's Disease, ALS (Lou Gehrig's Disease), and demyelinating conditions. We are also interested in applications outside the CNS particularly in musculoskeletal disease. Probing the molecular signals required for converting stem cells into specialized cell types in a culture dish will also provide novel insights into basic mechanisms of development. The lab is developing high throughput chemical and genetic screens to systematically address such questions in human embryonic stem cells.

#### ***Neural Differentiation of Embryonic Stem Cells***

We have recently succeeded in converting mouse and human embryonic stem cells into specific types of brain cells. This technology can combine the power of mouse genetics with defined in vitro assays of neural development. Manipulation of the embryonic stem cell genome to carry dominant and recessive mutations is the basis for the current revolution in mammalian genetics. Our techniques will allow dissection of the specific function of such mutations in brain development, including mutations that would lead to an early embryonic death during in vivo development. Study of developmental processes in a culture dish will also provide a platform for developing high-throughput functional genomic approaches.

#### ***Mesenchymal Differentiation of Human Embryonic Stem Cells***

Novel protocols in the lab allow the isolation of multipotent mesenchymal precursors from human embryonic stem cells. Such human embryonic stem cell derived mesenchymal precursors can be expanded in vitro or differentiated into fat, cartilage, bone and skeletal muscle cells. We are currently assessing the therapeutic potential of human stem cell derived mesenchymal precursors in musculoskeletal disease.

### ***Nuclear Transfer and Parthenogenetic Stem Cells***

Cell therapy raises the issue of immunocompatibility between transplanted cells and recipient. An ideal cell source would contain DNA that matches that of a potential patient. In animal models, we are developing 3 distinct strategies toward this goal:

1. Nuclear reprogramming (via nuclear transfer into an oocyte) allows the generation of mouse embryonic stem cell lines from adult somatic cells. In a collaborative effort, we have produced a large number of such lines from adult somatic cells. We have shown that such ntES cells can be coaxed into many specialized cell types, including midbrain dopamine neurons. We have also demonstrated the function of ntES derived dopamine neurons in vivo upon transplantation into a mouse model of Parkinson's Disease.
2. Parthenogenesis allows the generation of pluripotent ES-like stem cells via activation of an unfertilized egg. In a collaborative effort, our lab has demonstrated the derivation and differentiation of parthenogenetic stem from an adult monkey into a variety of specialized cell types.
3. Future research will be directed toward identifying the molecules responsible for the reprogramming of adult cells (e.g., during nuclear transfer). Such molecules could be used to reprogram adult cells directly without the need for nuclear transfer.

### ***Neural Stem Cells***

We have described how midbrain rat stem cells and human brain stem cells can be proliferated in culture and differentiated into dopamine neurons that, upon transplantation, restore behavioral deficits in a rat model of Parkinson's Disease. Studies directed toward the clinical application of CNS stem cells in Parkinson's Disease are underway.

Additional ongoing studies demonstrate that similar techniques allow the successful derivation of GABA neurons from brain stem cells with subsequent transplantation into animal models of Huntington's Disease.

### ***High Throughput Screens in Human Embryonic Stem Cells***

We have recently optimized techniques for hESC growth and differentiation under conditions suitable for high throughput chemical and genetic screens. Such technology will allow the identification of specific chemicals and genes that influence stem cell differentiation. This work is done in collaboration with the SKI High Throughput Screening core facility, headed by Hakim Djaballah.

### ***Studer Lab Pursuing New Applications for Human Embryonic Stem Cells***

Recent funding from [www.projectals.org](http://www.projectals.org) and the [www.alsa.org](http://www.alsa.org) opens up new opportunities to explore the use of human embryonic stem cells in the treatment of ALS.

Recent Support from the Kinetics Foundation has been essential in studies comparing the potential of various hES cell lines for the future treatment of Parkinson's disease. Currently over 20 hESC lines are being tested.

New funding from the Starr Foundation will allow the Studer lab to expand current efforts and to explore novel uses of embryonic stem cells in animal models of disease. The new Tri-Institutional Stem Cell Initiative will also create new Core-Facilities for Human Embryonic Stem Cell Research that should greatly accelerate the efforts in the lab.

## 5. Annemarie Opprecht Parkinson Award 2012

### Lorenz Studer, Projekte

#### Therapeutic Cloning and Parthenogenetic Stem Cells

##### *Nuclear Transfer ES Cells*

Therapeutic cloning offers a strategy for generating autologous (genetically identical to graft recipient) cells for transplantation therapy. We have previously shown that such embryonic stem cells can be generated in mice via nuclear transfer (ntES stem cells).

Donor nuclei can be obtained from an adult animal (e.g., after a tail biopsy of the mouse). ntES stem cells appear to have the same stem cell properties as regular ES cells but are genetically matched to the adult donor animal. These cells can be subsequently coaxed into specialized cell types, such as midbrain dopamine neurons (cells lost in Parkinson's Disease). We currently attempt a proof-of-principle application of therapeutic cloning by curing Parkinsonian mice with stem cells derived via nuclear transfer from the mouse's own tail.

##### *Parthenogenetic Stem Cells*

Parthenogenesis allows the generation of pluripotent ES-like stem cells via activation of an unfertilized egg cell. This is a process that does not require fertilization or any contribution of sperm. The genomic DNA of the resulting cells is completely maternally derived. Our lab has demonstrated the neural differentiation potential of such cells. Parthenogenetic stem cells could serve as an alternative source for autologous cell therapy.

Parthenogenetic stem cells are also an interesting tool to elucidate the role of imprinting during development, as these cells lack paternal imprinting. Current studies address the in vivo function of parthenogenetic stem cells after transplantation in animal models of neurodegenerative disease.

#### Neural Stem Cells

##### *Derivation of Neurons and Applications in Brain Repair*

We have described how stem cells, derived from both rat and human midbrain, can be proliferated and differentiated into dopamine neurons in culture. Transplantation of such stem cell-derived dopamine neurons into the brain can improve behavioral symptoms in animal models of Parkinson's Diseases.

Similar strategies are now being pursued for generating other neuronal and glial cell types, including GABA neurons and oligodendrocytes. These cell types will be tested in animal models of Huntington's Disease and demyelination.

##### *Lowered Oxygen Culturing*

We have described in vitro ambient oxygen concentration as a critical parameter in CNS stem cell culture. Standard tissue culture is carried out at 20 percent, whereas oxygen concentrations in the brain are dramatically lower. Reducing ambient oxygen levels to 2 to 4 percent (physiological range in vivo) leads to increased CNS precursor cell proliferation and survival, and affects neuronal subtype choice. Lowered oxygen culturing might be an important parameter for in vitro studies, using primary tissue. Current studies address the use of lowered oxygen in embryonic stem cell culture.

***Regional Specification***

Long-term proliferation of midbrain CNS precursors leads to changes in the expression of regional patterning genes with subsequent loss of dopaminergic differentiation. We are using differential gene expression profiling to further characterize these changes. Identification and re-introduction of the key molecules involved in regional patterning might allow for rescue of dopaminergic differentiation in long-term cultured stem cells. Similar assays are used to describe maintenance or loss of regional identity in precursor and stem cells from other brain regions.

**Neural Differentiation of Embryonic Stem Cells*****Mouse Embryonic Stem Cells***

The generation of unlimited numbers of dopamine neurons from mouse embryonic stem cells can be achieved in a multi-step differentiation protocol, allowing the sequential generation of embryonic stem cells, embryoid bodies, early ectodermal cells, proliferating CNS precursors, and differentiated neurons and glia. Alternatively, neural induction and directed differentiation into various neuronal and glial cell types can be achieved by co-culture with bone marrow-derived stromal feeder cell lines such as MS5 (see image)

***Human Embryonic Stem Cells***

We have developed protocols for the efficient generation of neural progeny from various human ES cell lines. Neural induction can be achieved with several strategies, such as the formation of embryoid bodies followed by growth in serum-free medium; co-culture with stromal feeder cells; or via overgrowth and manual selection. Specific types of neurons can be induced by exposure to factors that control the development of specific brain regions of interest.

Other proteins (cytokines) can be used to bias differentiation into glial versus neuronal cell types. Genetic modification of human ES cells will allow introduction of foreign genes for cell labeling in transplantation studies or for the local production of therapeutic proteins.

## 5. Annemarie Opprecht Parkinson Award 2012

### **Parkinson Schweiz: Wer wir sind und was wir tun**

Parkinson Schweiz ist die landesweit tätige Fachorganisation im Dienste der Patienten und ihrer Angehörigen. Sie wurde 1985 von Neurologen gegründet und hat mehr als 5500 Mitglieder. Parkinson Schweiz ist unabhängig, politisch und konfessionell neutral, gemeinnützig und von der Stiftung ZEWÖ zertifiziert. Parkinson Schweiz finanziert sich über Mitgliederbeiträge, Spenden, Gelder der öffentlichen Hand und Sponsoring. Die Dienstleistungen werden grundsätzlich in drei Schweizer Landessprachen (Deutsch, Französisch, Italienisch) erbracht. Dazu unterhält die Vereinigung neben der Geschäftsstelle in Egg ZH Regionalbüros in Lausanne VD und in Mezzovico TI.

#### ***Philosophie und Mission***

Parkinson Schweiz informiert, unterstützt, berät und begleitet die rund 15 000 Betroffenen in der Schweiz mit dem Ziel, dass diese trotz ihrer Erkrankung eine möglichst hohe Lebensqualität erlangen können. Als Fachstelle für Parkinson sind wir dabei ein verlässlicher Partner für alle Betroffenen. Aus dem Verständnis heraus, dass eine effiziente Unterstützung der Patienten nur möglich ist, wenn auch ihr soziales Umfeld miteinbezogen wird, verstehen wir uns gleichzeitig auch als zentrale Anlaufstelle für Fachpersonen und die Öffentlichkeit. Da sich Parkinson durch die fortschreitende Überalterung der Gesellschaft in den kommenden Jahren zu einer grossen gesundheitspolitischen Herausforderung entwickeln wird, nimmt die Bedeutung unseres Engagements kontinuierlich zu.

#### ***Die fünf Säulen unserer Arbeit***

##### Information

- Durchführung von Informationstagungen
- Publikation von Broschüren, dem Magazin PARKINSON und der Website [www.parkinson.ch](http://www.parkinson.ch)
- Aufklärung der Öffentlichkeit und Medienarbeit

##### Beratung

- für Betroffene (Patienten und Angehörige), Arbeitgeber, Fachleute aus Medizin und Pflege
- Gratis-Hotline PARKINFON 0800 80 30 20, der direkte Draht zum Neurologen

##### Selbsthilfe

- Förderung von mehr als 70 Selbsthilfegruppen; Weiterbildung der Leitungsteams

##### Aus- und Weiterbildung

- Seminare und Kurse für Betroffene, Angehörige und die Mitglieder der Selbsthilfegruppen
- Aus- und Weiterbildung von Fachpersonen aus Medizin, Pflege und Therapie

##### Forschungsförderung

- Finanzielle Unterstützung ausgewählter Forschungsprojekte zum Thema Parkinson.

#### ***Kontakt:***

Dr. Jörg Rothweiler, Leiter Kommunikation, Parkinson Schweiz,  
Gwerbestrasse 12a, Postfach 123,  
CH-8132 Egg, Tel. 043 277 20 77, Fax 043 277 20 78, E-Mail: [presse@parkinson.ch](mailto:presse@parkinson.ch)

Bureau romand [Chemin des Charmettes 4, 1003 Lausanne, tél./fax 021 729 99 20, info.romandie@parkinson.ch](http://Chemin des Charmettes 4, 1003 Lausanne, tél./fax 021 729 99 20, info.romandie@parkinson.ch)  
Ufficio Svizzera italiana [Piazzora da Vira, 6805 Mezzovico, tel. 091 755 12 00, fax 091 755 12 01, info.ticino@parkinson.ch](http://Piazzora da Vira, 6805 Mezzovico, tel. 091 755 12 00, fax 091 755 12 01, info.ticino@parkinson.ch)



## 5. Annemarie Opprecht Parkinson Award 2012

### Die Parkinsonkrankheit: das Wichtigste in Kürze

Parkinson ist eine der häufigsten neurologischen Erkrankungen weltweit. Aufgrund der zunehmenden Überalterung der Bevölkerung muss künftig mit einem deutlichen Anstieg der Zahl der Betroffenen gerechnet werden.

#### **Antworten auf die häufigsten Fragen**

##### **Was löst Parkinson aus?**

- Die Ursache der Erkrankung ist bislang ungeklärt.

##### **Wie häufig ist Parkinson?**

- Weltweit gibt es rund 4,1 Millionen Parkinsonpatienten. Laut Studien wird ihre Zahl bis ins Jahr 2030 auf rund 8,7 Millionen steigen. In der Schweiz leben rund 15 000 Betroffene.

##### **Wer ist betroffen?**

- Parkinson betrifft etwa ein Prozent der über 60-Jährigen und rund drei Prozent der über 80-Jährigen. Bis zu 20 Prozent der Patienten sind bei der Diagnose jünger als 60 Jahre. Männer und Frauen sind etwa gleich oft betroffen.

##### **Ist Parkinson vererblich?**

- Es gibt eine vererbliche Variante. Sie ist aber sehr selten.

##### **Ist Parkinson ansteckend?**

- Nein.

##### **Sterben die Patienten früh?**

- Nein. Die Lebenserwartung der Erkrankten ist etwa gleich hoch wie bei gesunden Menschen.

##### **Wie wird Parkinson behandelt?**

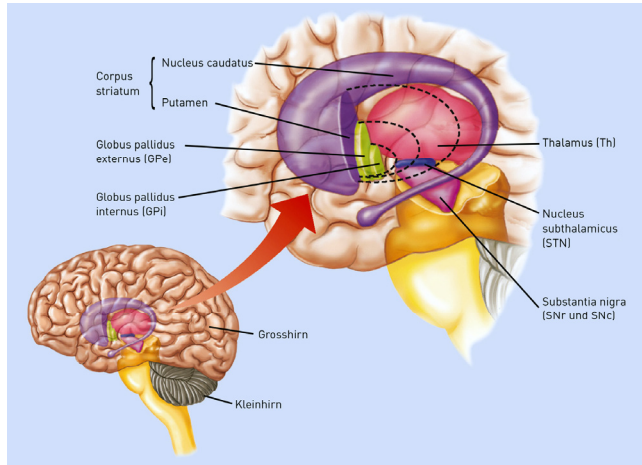
- Medikamente, Begleittherapien, manchmal Operationen.

#### **Was im Körper geschieht**

Die Parkinsonkrankheit (Morbus Parkinson) ist eine neurodegenerative Erkrankung, bei der es zu einem fortschreitenden Untergang von Nervenzellen im zentralen Nervensystem, besonders im Gehirn, kommt. In den ersten Krankheitsjahren steht der Untergang der für die Produktion des Botenstoffes Dopamin verantwortlichen Nervenzellen in der Substantia nigra (Schwarzer Kern, im Mittelhirn gelegen) im Vordergrund. Der resultierende Dopaminmangel führt zu diversen Störungen der Motorik. Diese können durch die Behandlung mit L--Dopa, der Vorläufersubstanz von Dopamin, gut behandelt werden.

Schon früh im Krankheitsverlauf sterben aber auch in anderen Regionen des Gehirns Nervenzellen ab, die nichts mit der Produktion von Dopamin zu tun haben. Dies führt zu einer Reihe von Symptomen (z. B. vegetative Störungen, Schmerzen, Schlafstörungen, psychische Symptome), die mit fortschreitender Krankheitsdauer für die Patienten immer belastender werden. Für diese Symptome sind die Behandlungsmöglichkeiten derzeit leider noch stark eingeschränkt.





### **Die Basalganglien**

*Die Basalganglien bilden einen Teil des extrapyramidalen motorischen Systems und umfassen mehrere Kerne, darunter den Nucleus subthalamicus (STN), den Globus pallidus (GPe und GPi) sowie die Substantia nigra (SN) mit den beiden Unterbereichen Pars compacta (SNc) und Pars reticulata (SNr). Abnormalitäten im Bereich dieser Kerne führen zu Bewegungsstörungen.*

### **Die wichtigsten Symptome**

Eine Besonderheit der Parkinsonerkrankung ist die stark individuelle Ausprägung der Symptomatik, welche sich von Patient zu Patient stark unterscheiden kann. Die im Folgenden aufgelisteten Symptome treten also nicht bei jedem Patienten auf!

#### **Unbeweglichkeit (Bradykinese, Akinese)**

- Flüssige Bewegungsabläufe fallen zunehmend schwerer. Die Patienten bemerken dies anfänglich beim Schreiben, bei handwerklichen Tätigkeiten oder beim Spielen eines Musikinstruments. Mit fortschreitender Krankheit nimmt die Akinese zu.

#### **Steifheit (Rigor)**

- Der Spannungszustand der Muskulatur ist bei Parkinson ständig erhöht, vor allem an den Gliedmassen, die daher zittern oder eingeschränkt beweglich sind. Die Patienten haben den Eindruck, ihre Glieder seien «wie gelähmt», und leiden häufig unter schmerzhaften Krämpfen.

#### **Zittern in Ruhe (Ruhetremor)**

- Das Ruhезittern, welches bei rund zwei Dritteln der Betroffenen auftritt, ist anfangs in der Regel einseitig ausgeprägt. Die Arme sind meist stärker betroffen als die Beine. Bei gezielten Bewegungen verschwindet das Zittern.

#### **Haltungsinstabilität (posturale Instabilität)**

- Der aufrechte Gang und das Gleichgewicht des Menschen werden durch ein komplexes Regulationssystem sichergestellt, in welchem die automatisch ablaufenden Halte- und Stellreflexe eine zentrale Rolle einnehmen. Da diese Automatismen bei der Parkinsonkrankheit gestört sind, fällt es den betroffenen Personen mit fortschreitender Krankheit immer schwerer, die aufrechte Haltung beizubehalten. Eine gefährliche Folge dieser Beeinträchtigung sind Stürze.

#### **Weitere Symptome**

- Weitere, verhältnismässig häufige Symptome der Parkinsonkrankheit sind psychische Veränderungen (z.B. Depressionen), Anomalien des Schlaf-Wach-Rhythmus und Störungen des vegetativen Nervensystems (Blutdruck- und Temperaturregulation, Verdauung). In fortgeschrittenem Stadium sind Hirnleistungsstörungen keine Seltenheit.

### ***Aktuelle Behandlungsmöglichkeiten***

Solange die Ursachen nicht bekannt sind, kann es auch keine Behandlung geben, die das Übel an der Wurzel packt und damit eine Heilung bringt. Im Gegensatz zu vielen anderen Krankheiten des Nervensystems gibt es jedoch wirksame Medikamente, welche das Krankheitsbild über Jahre hinweg so unter Kontrolle halten, dass ein weitgehend normales Leben möglich ist.

Neben Medikamenten, mit denen das Gleichgewicht der Botenstoffe wieder hergestellt werden soll, benötigen die Betroffenen begleitende Massnahmen wie Physiotherapie, Ergotherapie, Logopädie (Sprachtherapie), Entspannungstherapien und gelegentlich auch Psychotherapie, beispielsweise gegen Depressionen. Da die Krankheit schleichend verläuft, muss eine Behandlung nicht unmittelbar nach der Diagnose erfolgen. Patient und Arzt besprechen vielmehr gemeinsam, wann die Beeinträchtigung durch die Symptome so gross ist, dass Medikamente eingesetzt werden müssen.

Mit hochpräzisen Eingriffen wie der Tiefen Hirnstimulation (Hirnschrittmacher) kann eine Verbesserung des Gesundheitszustandes erzielt werden. Allerdings sind nur sehr wenige Patienten für einen solchen Eingriff geeignet. Zudem können auch Operationen das Fortschreiten der Parkinsonkrankheit nicht verzögern oder gar zum Stillstand bringen.

### ***Historisches***

Der Londoner Arzt und Apotheker James Parkinson (1755 – 1824) beschrieb 1817 in seinem «Essay on the Shaking Palsy» erstmals die Symptome der Erkrankung. 1884 benannte der französische Neurologe Prof. Jean Marie Charcot die Krankheit nach ihrem Ent-decker «Morbus Parkinson».

### ***Prominente Parkinsonpatientinnen und -patienten***

Muhammed Ali, Jassir Arafat, Leonid Breschnew, Johnny Cash, Salvador Dali, Otfried Fischer, Michael J. Fox, Raimund Harmstorf, Katharine Hepburn, Adolf Hitler, Peter Hofmann, Deborah Kerr, Papst Johannes Paul II., Vincent Price, Manfred Rommel, Theodore Roosevelt, Mao Tse-tung, Wilhelm Freiherr von Humboldt, Deng Xiaoping.

Mehr Informationen finden Sie auch auf unserer Website [www.parkinson.ch](http://www.parkinson.ch)

## 5. Annemarie Opprecht Parkinson Award 2012

### CV Lorenz Studer, ausführliche Version

- Name:** Lorenz Peter Studer
- Date of Birth:** March 5, 1966
- Place of Birth:** Solothurn, Switzerland
- Nationality:** Swiss (Permanent Resident of the US)
- Marital Status:** married, two children
- Languages:** English, German, French
- Education:**
- College:** 1985 Matura degree type B, Olten, Switzerland
- Medical School:** 1987 Cand. Med. degree, Univ. of Fribourg, Switzerland  
1991 Medical Degree, Univ. of Bern, Switzerland  
1994 Doctorate in Neuroscience, University of Bern, Switzerland. *"NGF increases neuritic complexity of cholinergic interneurons in organotypic cultures of neonatal rat striatum"*.
- Postdoctoral Training:**
- 1994 – 1995 Postdoctoral fellow Department of Neurosurgery, Bern
- 1996 – 1998 Postdoctoral fellow, Laboratory of Molecular Biology, NINDS, NIH, Bethesda, Maryland
- Positions and Appointments:**
- 1992 – 1993 Research appointment, Dept. of Physiology (headed by Prof. H.-R. Lüscher) , Bern, Switzerland
- 1993 – 1995 Scientific residency, Dept. of Neurosurgery (headed Dr. C. Spenger and Prof. R.W. Seiler), INSELSPITAL, Bern, Switzerland

1996 – 1998	Research fellowship, Laboratory of Molecular Biology (headed by Dr. Ron McKay), NINDS, NIH, Bethesda, Maryland
1998 – 1999	Visiting associate, Laboratory of Molecular Biology (headed by Dr. Ron McKay), NINDS, NIH, Bethesda, Maryland
2000 - 2003	Assistant Member, Sloan-Kettering Institute, Cellular Biochemistry and Biophysics Program (Chair: Dr. J. Rothman).
2003 - 2005	Assistant Member, Sloan-Kettering Institute, Developmental Biology Program (Chair: Dr. K. Anderson)
2006 - 2009	Associate Member, Sloan-Kettering Institute, Developmental Biology Program (Chair: Dr. K. Anderson)
2009 – current	Member, Sloan-Kettering Institute, Developmental Biology Program (Chair: Dr. K. Anderson)
2010 – current	- Director of the SKI Center for Stem Cell Biology

Current positions:

2009 – current	- Attending, Division of Neurosurgery, Memorial Sloan Kettering Cancer Center.
2009 – current	- Professor in Developmental Neurobiology, Cornell University, Graduate Program in Neuroscience
2009 – current	- Member, Sloan-Kettering Institute, Developmental Biology Program (Chair: Dr. K. Anderson).
2007 - current	- Academic Supervisor/Principle Investigator of SKI human ES cell core facility (Starr Foundation and NYSTEM funded facility; awarded > \$ 6 million in total grant support)
2010 – current	- Director of the SKI Center for Stem Cell Biology

### **Scientific and Medical Societies:**

- Society for Neuroscience (regular member, since 1994)
- Cell Transplantation Society (one of the founding members, 1991)
- Network for Cerebral Transplantation and Restoration (NECTAR) (foreign member, since 1996)
- International Society for Stem Cell Research (regular member since 2003)
- New York Academy of Sciences (since 2004)
- New York Stem Cell Foundation (Medical Advisory Board, since 2008)

### **Honors, awards and patents:**

1995	First Swiss clinical trial of neural transplantation
1996	Fellowship award, Swiss National Science Foundation.
1997	Fellowship award, National Institutes of Health.
1999	Travel award, NINDS.
1999	U.S. Patent 6,787,356 "Cell expansion system for neural transplantation"
	U.S. Patent US Pat. 6,610,540 "Low Oxygen Culturing of CNS Progenitor Cells"
2000	US Patent 10/127,740 "Derivation of midbrain DA neurons from ES cells"
2001	MJ Fox research award
2001	Patent WO02086073 "Generation of differentiated tissue from nuclear transfer embryonic stem cells and methods of use"
2002	MJ Fox Research PD Cell Line Grant Award
2004	Patent application "Derivation of midbrain dopamine neurons from human ES cells.
2005	Patent application "Derivation of mesenchymal precursors and their derivatives from human ES cells
2005	<u>Boyer Award in Basic Research</u>
2007-2012	President Steering Committee, Tri-institutional Stem Cell Initiative
2012-curr.	Member Steering Committee, Tri-institutional Stem Cell Initiative
2007-2011	Member, Faculty of 1000
2008	Provisional Patent application: "HTS assay in human embryonic stem cells"
2008	Provisional Patent application: SK1324 – "Large DNA fragment transgenesis of human embryonic stem cells.
2009/10	Patent 2012/0094,381, Methods of neural conversion of human embryonic stem cells
2010 –curr	Founding Director, <a href="#">Center for Stem Cell Biology (CSCB)</a> at the Sloan-Kettering Institute,
2010/11	WO/2010/141622 Methods to isolate Embryonic Stem Cells Directed to Forebrain Interneuron Fate
	Patent PCT/US2011/37179 "Methods of nociceptor differentiation of human embryonic stem cells and uses thereof"
2011/12	PCT/US 61/555,238 Floor Plate Derived Midbrain Dopamine (DA) Neurons For Therapeutic and Research Use
2012	<u>Annemarie Opprecht Award</u> ( <a href="http://www.opprecht-foundation.org/">http://www.opprecht-foundation.org/</a> )

**Reviewer/Consultant/Committees:**

1996 – current

*Adhoc Reviewer* for Nature, Science, Cell, Nature Biotechnology, Nature Medicine, Nature Neuroscience, New England Journal of Medicine, Cell Stem Cell, PLoS Medicine, PLoS Biology, Journal of Neuroscience, PNAS, Development, Journal of Cell Biology, Stem Cells, Genes & Development, European Journal of Neuroscience, Journal of Neuroscience Methods, Brain, Journal of Experimental Medicine, Experimental Neurology, Journal of Clinical Investigation, Brain Research, Developmental Neuroscience, Journal of Neurochemistry, Journal of Neuroscience Research, Molecular Cellular Neuroscience, FASEB and others.

2000 *Reviewer* for Office of External Reviews, Dept. of Veterans Affairs;

2001 *Adhoc reviewer* on NINDS/NIH study section RFA-NS-02-006 “Fast track grants for Parkinson’s Disease Research”, Consultant: Neuronix, Malvern, PA

2002 *Adhoc Reviewer* for NINDS/NIH study section (NSD-B), for Swiss National Science Foundation and for Joseph Steiner Foundation for Cancer Research

2003 *Adhoc Reviewer* NINDS/NIH study section (NSD-B) and NINDS/NIH study section (ZNS1-SRB-E), adhoc consultant for MJ Fox Foundation.

2004 *Permanent member* NSD-B starting June 2004. *Adhoc reviewer* Developmental Biology Grants MJ Fox Foundation, *Consultant* for FLENI (Fundación para la Lucha contra las Enfermedades Neurológicas de la Infancia), Argentina, *Adhoc reviewer* Juvenile Diabetes Research Foundation & Biomedical Research Concl, Singapore, *Adhoc Consultant* AMDeC Embryonic Stem Cell Research Initiative.

2005 *Permanent member* NSD-B. *Adhoc reviewer* Developmental Biology Grants MJ Fox Foundation, *consultant*, MJ Fox Foundation, *Member Steering Committee* Tri-institutional Stem Cell Initiative, SKI/Rockefeller/Cornell University.

2006 *Permanent member* NSD-B. *Member and President Steering Committee* Tri-institutional Stem Cell Initiative, SKI/Rockefeller/Cornell University; *Reviewer* for National Science Foundation; *Member of Review Committee* for Stem Cell Research Foundation; *Adhoc reviewer* Developmental Biology Grants MJ Fox Foundation; *Reviewer* for Schweizer Paraplegiker Stiftung, Switzerland;

2007 *Permanent member* NSD-B

Reviewer NIH/NIGMS Program Projects for Research on hESCs

Co-Chair NIH Blueprint on hESCs and Neural Differentiation

*Member and President Steering Committee* Tri-institutional Stem Cell Initiative, SKI/Rockefeller/Cornell University

Medical Advisory Board, New York Stem Cell Foundation.

- 2008 *Permanent member* NSD-B  
 Adhoc member DEV-2  
*Member and President Steering Committee* Tri-institutional Stem Cell Initiative, SKI/Rockefeller/Cornell University  
*Member Review panel* California Institute of Regenerative Medicine  
*Member of Executive Committee* of the Center for Cell Engineering MSKCC  
 Michael J. Fox Foundation, Expert Reviewer on RFA for companies involved in PD research.  
 Medical Advisory Board, New York Stem Cell Foundation.
- 2009 *Permanent member* DEV-2 NIH study section (starting Sept 09).  
 President Steering Committee for Tri-institutional Stem Cell Initiative (Rockefeller U., Cornell U., SKI),  
 Medical Advisory Board NYSCF,  
 NYSCF reviewer for fellowship applications,  
 Adhoc reviewer NIH/NIBPB, RFA Enabling technologies in stem cell research,  
 Reviewer MJ Fox Foundation for PD research - TDI program.
- 2010 *Permanent member* DEV-2 NIH study section (starting Sept 09).  
 President Steering Committee for Tri-institutional Stem Cell Initiative (Rockefeller U., Cornell U., SKI),  
 Medical Advisory Board NYSCF,  
 NYSCF reviewer for fellowship applications,  
 NYSCF Jury Member, Innovator Awards Program for Early Career Investigators,  
 CIRM review panel for Leadership Awards.
- 2011 *Permanent member* DEV-2 NIH study section (starting Sept 09).  
 Consultant: Vertex Pharmaceuticals  
 President Steering Committee for Tri-institutional Stem Cell Initiative (Rockefeller U., Cornell U., SKI);  
 Medical Advisory Board NYSCF;  
 NYSCF Jury Member, Robertson Investigator Award in Stem Cell Biology;  
 NYSCF Jury Member, Robertson Investigator Awards in Neuroscience;  
 Advisory Council, Allen Brain Institute;  
 CIRM review panel for Leadership Awards.  
 Member NIMH stem cell advisory panel - NIMH Center Repository supporting stem cell research
- 2012 *Permanent member* DEV-2 NIH study section (starting Sept 09).  
 President / Member Steering Committee for Tri-institutional Stem Cell Initiative (Rockefeller U., Cornell U., SKI);  
 Medical Advisory Board NYSCF;  
 NYSCF Jury Member, Robertson Investigator Award in Stem Cell Biology;  
 NYSCF Jury Member, Robertson Investigator Awards in Neuroscience;  
 CIRM review panel " Basic Biology IV".  
 External Advisory board: NYU stem cell program

**Invited lecturer:**

- 1994 - Regeneron Pharmaceuticals, Tarrytown, NY (1994)
  - Molecular Mechanisms of Neurodegenerative diseases, Ochos Rios, Jamaica
  
- 1995 - Molecular Mechanisms of Neurodegenerative diseases, Ochos Rios, Jamaica
  
- 1997 - Society for in vitro biology and FDA, Laurel, MD
  
- 1998 - European Society for Stereotactic and Functional Neurosurgery, Freiburg, Germany
  
- 1999 - International Workshop for precursor cell biology in Tokyo, Japan
  - NECTAR meeting: „The future of neural grafting“ Santorini, Greece
  - Grand Rounds Lecture, Cornell University, New York, NY
  
- 2000 - Swedish Movement Disorder Society, “Stem cells in future treatment of movement disorders” SWEMODIS, Lund, Sweden
  - Neural Workshop, Verbier Switzerland
  - Surgical Research Conference, MSKCC, New York
  - Progress in Neuroscience Seminar, Cornell University, Weill Medical School, New York
  - Gordon Research Conference “Signal Transduction by engineered extracellular matrices” Tilton, NH
  - Neuropathology 2000 Congress, Birmingham, UK
  - NECTAR annual meeting (2000) in Hannover, Germany
  - Neuronyx Biotech, Malvern, PA
  
- 2001 - Advanced Cell Technology, Worcester, MA
  - Progress in Stem Cell Biology, Klenk Symposium, Köln, Germany,
  - Engineering Tissue Growth, Pittsburgh, PA
  - Harvard Medical School, McLean Hospital, Belmont
  - ARNMD, Annual Conference: Stem Cells for a New Clinical Neuroscience, New York City, NY
  - 2<sup>nd</sup> Annual Conference on Regenerative Medicine, Cloning and Stem Cell Biology – “Rebuilding the Human Body”, Washington, D.C.



- 2002 - Institute for Molecular Pathology, Vienna, Austria “The Stem Cell Challenge”
- Developmental Neuroscience Seminar Series, Rockefeller University, New York City
  - “Cellular and Molecular Treatments of Neurological Diseases“, Harvard University, Cambridge, MA
  - Columbia University, Pathology Department and Taub Institute, New York City
  - “Seminars in Neurobiology” Biozentrum, Basel, Switzerland
  - Annual Neuroscience Meeting of Japan: Symposium "Differentiation/regulation of ES and neural stem cells for application to neural transplantation"; Tokyo, Japan.
  - Association of the Bar of the City of New York. Panelist on Stem Cell Research: Ethics, Law and Public Policy
  - Society for Neuroscience, Orlando, FL, Speaker at Neural Disease Workshop on Stem Cells.
  - NECTAR annual meeting on Neural Transplantation, Amsterdam, Netherlands.
- 2003 - Neural Disease Workshop, Verbier, Switzerland
- National Institute of Drug Abuse, NIH Gaithersburg, “Stem Cells-Opportunities for Drug Abuse Research”
  - Stanford University, Department of Immunology & Microbiology
  - Michael J. Fox Cell Transplantation Summit, Menlo Park, CA.
  - MSKCC, Ethics committee “Stem Cell Research and its Ethical Implications”
  - Michael J Fox Meeting: Developmental Biology in Parkinson’s disease research.
  - Banbury Meeting on Pluripotent Stem Cells, Cold Spring Harbor Laboratories
  - Stem Cell Symposium, Hanyang University, Seoul, Korea
- 2004 - Surgery Grand Rounds, MSKCC
- Dean’s hour lecture, Weill Medical College of Cornell University
  - Grand Rounds, Neurology, New York Hospital
  - American Society for Gene Therapy, Minneapolis, Minnesota
  - Pittsburgh, Development Center: Frontiers in Human Embryonic Stem Cell Research
  - Hertie Opening Symposium, Hertie-Institute, University of Tubingen, Germany
  - 4th Forum of European Neuroscience, Lisbon, Portugal
  - Santa Cruz Conference on Developmental Biology
  - NY State, Dept. of Health, Ethics Advisory board, New York, NY
  - Innovation: The Future of Ophthalmology, Four Seasons Hotel, New York, NY
  - Stem Cell Lecture Series, Weill Medical College of Cornell University, Uris Auditorium
  - Lund Strategic Center For Stem Cell Biology, Stem Cell Frontiers Seminar Series Lecture
  - NECTAR meeting, Brussels, Belgium

- 2005
- Carnegie Institution of Washington, Baltimore, MD
  - Winter Brain Research Conference, Breckenridge, CO
  - New York Academy of Sciences, Genomic Medicine Interest Group (Symposium)
  - University of Geneva, Switzerland
  - Translational Seminar Series, MSKCC
  - Challenges Associated with Cell Differentiation, United States Army Neurotoxin Exposure Treatment Research Program (NEPTRP). Development of Inventory of Parkinson's Disease and Parkinsonism Treatment and Research Meeting Series, Tampa, Florida
  - 130th American Neurological Association Meeting, San Diego
  - Udall Parkinson's Disease Research Center Seminar Series, Harvard Medical School
  - Overcoming Stem Cell Research Challenges, IBC's International Conference, San Diego, CA
  - Hertie FENS Winterschool in Neurosciences, Innsbruck, Austria
  - Banbury Conference. Cold Spring Harbor, Banbury Center "THE BIOLOGY AND PRACTICE OF MAMMALIAN CLONING: A REASSESSMENT"
- 2006
- Mt. Sinai School of Medicine, New York, Black Family Stem Cell Institute Lecture World Parkinson Congress, Washington D.C., Session Chair on Stem Cells in PD.
  - Banbury Conference, Cold Spring Harbor, Banbury Center "PARKINSON'S DISEASE: INSIGHTS FROM GENETIC AND TOXIN MODELS"
  - George Washington University (Sally Moody), GWUMC Stem/Progenitor Cell Research Interest Group, Washington D.C.
  - Nature Magazine, New York, NY. Roundtable on Stem Cells
  - Duke University Stem Cell Workshop (organized by Brigid Hogan) Keynote lecture
  - Society for Neuroscience Meeting in Atlanta. Speaker at Symposium on "Generation of Midbrain Dopamine Neurons from Embryonic Stem Cells for Transplantation in Parkinson's disease"
  - Harvard University, IV. Conference on "Cellular and Molecular Treatments of Neurological Diseases" in Cambridge, Massachusetts.
  - ISSCR Annual Meeting in Toronto, Canada, Plenary Speaker in session on "Establishment of the Lineage"
  - Movement Disorders Society, Annual Meeting, Kyoto, Japan
  - National Academy of Sciences, Washington D.C. Human Embryonic Stem Cell Guidelines
  - Cornell University, New York, NY. Neurology Grand Rounds
  - First Annual Translational Stem Cell Research Conference, NYSCF, held at Rockefeller University, New York, NY

- 2007 - New York University, Skirball Institute (Ruth Lehman),
- Columbia University (F. Doetsch, H. Wichterle), Stem Cell Lecture Series
  - CNS Regeneration Meeting, Boston, Biosymposium Series
  - Michael J. Fox Foundation Symposium, London, UK. Stem cells and Parkinson's disease.
  - EuroSTEM lecture, Karolinka Insitute, Sweden
  - Nobel symposium, Stockholm, Sweden
  - 12th International Symposium on Neural Regeneration, Asilomar, CA
  - Harvard Stem Cell Institute, Symposium on dopamine neurons and stem cells (J. Macklis)
  - University of North Carolina, Chapel Hill (L. Reid)
  - American Thyroid Association, New York
  - Woods Hole Course on Human Embryonic Stem Cells
  - EuroSTELLS Workshop in Milano, Italy : "Challenges in Stem Cell Differentiation and Transplantation
  - Second Annual Translational Stem Cell Research Conference, NYSCF, held at Rockefeller University, New York, NY
  - Banbury Meeting "Cell Transplantation as a Therapy for Parkinson's Disease"
- 2008 - Adler Foundation Symposium (F. Gage) : "Stem Cells: Endogenous and exogenous Applications, The Salk Institute, LaJolla, CA
- Connecticut Stem Cell Research retreat (L. Grabel); Keynote speaker, Wesleyan University, CT
  - First International Symposium on Human Embryonic Stem Cell Research, Evry-Paris, France.
  - Johns Hopkins University School of Medicine, The institute for cell engineering, Spring symposium 2008
  - 73rd CSHL Symposium on Quantitative Biology: " Control and Regulation of Stem Cells" Cold Spring Harbor Laboratories
  - Boehringer Ingelheim Fonds, International Titisee Conferences (R. Jaensich & T. Graf): "Differentiation, reprogramming and regeneration"
  - Banbury Center - Cold Spring Harbor workshop on Stem Cell Technologies
  - UCSF, Stem Cell Program, stem cell lecture series (A. Kriegstein)
  - Harvard University, Harvard Stem Cell Institute, Malkin-Symposium (D. Melton, K. Hochedlinger)
  - National Academy of Sciences: NATIONAL ACADEMIES HUMAN EMBRYONIC STEM CELL RESEARCH ADVISORY COMMITTEE; Symposium on Translation of Stem Cells into Clinical Stem Cell Therapeutics

## 2009 (selected)

- Abcam - Human Pluripotent Stem Cells symposium: interrogating disease and development – Dublin, Ireland
- Keystone Symposium - Neurodegenerative Diseases: New Molecular Mechanisms – Keystone, CO
- European School of Molecular Medicine Symposium: 'Reprogramming Cell Fate: Basic Biology and Medical Perspectives', Milano, Italy
- ES Tools, Winterschool, (Austin Smith, Oliver Brustle; organizers, Finland)
- Regeneron Pharmaceutical, Tarrytown, NY
- Albert Einstein College of Medicine, Stem Cell Series Lecture
- Developmental Origin of Neurological Disorders: From Neurogenesis to Circuit Formation (D. Epstein, M. Matisse; organizers); Baeza, Spain
- Burke, Medical Research Center, NY: Tuesday Lecture Series (G. Gibson)
- San Sebastian, Spain: Keynote Lecture on Symposium on "NEURONAL PLASTICITY AND REGENERATIVE MEDICINE IN PARKINSON DISEASE", Fundación Inbiomed
- Neurostemcell - Symposium: "Generation of transplantable mesDA and striatal precursors from stem cells for use in clinical applications" (A. Bjorklund, Elena Cattaneo, Austin Smith; organizers). Hydra – Greece
- Keystone Symposium: "Neurodegenerative Diseases: New Molecular Mechanisms"; (V. Dawson & D. Holtzman; organizers): Keystone, Colorado
- NYSCF: "Fourth Annual Translational Stem Cell Research Conference". Rockefeller University, NYC
- NYSTEM, 1st Symposium on Stem Cell Research, Albany, NY
- UCSF, 2nd International Stem Cell Symposium: "Frontiers in Neural Stem Cells" (A. Kriegstein, organizer)
- 2nd Southeast Stem Cell Workshop, University of Georgia
- World Parkinson Congress, Miami, FL

## 2010 (selected)

- National Institutes of Health, "Workshop on induced pluripotent stem cells" (Francis Collins, organizer)
- Rochester University - Grand Rounds in Neurology (Steve Goldman, organizer)
- Edinburgh, Scotland, Stem Cell Center Lecture (Ian Chambers, organizer)
- CIRM Annual Grantee Meeting, San Francisco, CA: Keynote lecture (Alan Trounson, organizer)
- University of Wisconsin, Madison, Stem Cell Lecture series (Su-Chun Zhang, organizer)
- Neurostemcell Meeting, Bellagio Italy, Plenary Lecture, (Elena Cattaneo & Anders Bjorklund, organizer)
- EMBL Symposium "Stem Cells in Regenerative Medicine and Cancer Conference", Heidelberg, Germany, (Ian Wilmot, Thomas Graf and Sten Eirik Jacobsen, and Claus Nerlov, organizers) - Plenary Lecture

- ESTOOLS meeting, Lisbon, Portugal (Austin Smith, organizer) - Plenary Lecture
- American Society for Stereotactic and Functional Neurosurgery (ASSFN), 2010, biennial Meeting, New York, NY - Plenary Lecture
- 8th Annual ISSCR meeting in San Francisco, CA - Plenary Speaker - (Sean Morrison & Irv Weissman, organizers)
- UKNSC Third Annual stem cell meeting, Nottingham, UK - plenary lecture - (Peter Andrews, organizer)
- CSHL Summer Stem Cell Course, Lecturer, (Ron McKay, organizer)
- Vanderbilt University, Stem cell lecture series
- Karolinska Institute, Stem Cell Course lecturer
- World Parkinson's Congress, Glasgow, UK
- The Royal Society, 350 Year Celebration, London, UK " Stem cells for research and therapy", Plenary Speaker (Ian Wilmot, organizer)
- Canadian Stem Cell Network Symposium, Calgary, Canada (S. Weiss, M. Rudnicki: organizers)
- Roche Basel Symposium: "The impact of small molecules on stem cell biology" (M. Rogers-Evans, organizer)

#### 2011 (selected)

- University of Southern California (USC), Eli and Edythe Broad Center for Regenerative Medicine and Stem Cell Research (Qilong Ying and Martin Pera, hosts)
- Keystone Symposium, New Mexico, "Stem Cell in Tissue Homeostasis" (Ronald D. McKay, Elaine Fuchs and Thea D. Tlsty; organizers).
- Cold Spring Harbor Symposium, Cold Spring Harbor Laboratories, NY. "Cell Engineering" (Michel Sadelain, Rudolf Jaenisch, Amy Wagers; organizers).
- Keystone Symposium, New Mexico, "The molecular and cellular basis for neurodegeneration" (Ted Dawson, Virginia Lee, Stephen Strittmatter; organizers)
- Society for Neuroscience, Symposium: "Human Cell Reprogramming and Its Application in Neurological Diseases" (G. Ming, M. Wernig; organizers).
- Bellagio, Italy, Neurostem cell consortium (A. Bjorklund, E. Cattaneo; organizers)
- Cold Spring Harbor Asia, Symposium, Suzhou Dushu Lake Conference Center. China. "Cellular Programs & Reprogramming" (H. Deng, N. Kim, R.D.G. McKay, R. A. Young).
- Albert Einstein College of Medicine, Bronx, NY
- Familial Dysautonomia Foundation: FD Day, NYU, New York, NY (F. Axelrod; organizer)
- NECTAR annual meeting, Cambridge, UK (R. Barker; organizers)
- 5th annual NJ Stem Cell Symposium, Keynote Lecture (Rutgers Stem Cell Research Center; organizer)
- New York Academy of Sciences, NYAS, New York, NY: "Patient-Specific Induced Pluripotent Stem Cells for the Study of Neurological Diseases"
- NY Genetic Diseases of Children Conference. (K. Fischbeck, L. Sturmann).

- NYSCF Discussion Panel. The Times Center, New York, NY. "Parkinson's Disease: How stem cell research will make a difference"
- Stanford University: Institute for Stem Cell Biology and Regenerative Medicine (M. Wernig; organizer)
- Pfizer / Neusentis Symposium. Cambridge, UK (R. McKernan: organizer)
- New Jersey Stem Cell Symposium. Bridgewater Marriott: Keynote lecture on Fifth Annual New Jersey Stem Cell Research Symposium.
- Vanderbilt University. Nashville TN, VUMC Seminar Series (A. Bowman, organizer)
- Rockefeller University: New York, NY. Seminars in clinical research

#### 2012 (selected)

- EMBL Conference: Stem Cells in Cancer and Regenerative Medicine, Heidelberg, Germany. (I. Wilmut and C. Nerlov: organizers)
- Swiss Stem Cell Network: Keynote address. Zurich, Switzerland
- Gordon Conference: Reprogramming Cell Fate. Galveston, TX (K. Plath, K. Hochedlinger; organizers)
- Fondation IPSEN; Programmed cells from basic neuroscience to therapy. Paris, France. (F.H. Gage & Y. Christen; organizers)
- Johnson & Johnson Stem Cell Day: New York, NY (Tri-institutional stem cell initiative; organizers)
- New York University / NYU stem cell program lecture series. (R. Lehmann organizer)
- Eppley Institute, University of Nebraska, (A. Rizzino; organizer)
- Keystone Conference: The Life of a Stem Cell: From Birth to Death. Squaw Creek, Olympic Valley, CA (M.A. Goodell, R. Lehmann and T.A. Rando; organizers)
- Neurostem cell symposium, London, UK "Designing Phase I Clinical Trial with Transplantation of Candidate Cell Line in Parkinson's Disease. the first stem cell based clinical trial in PD" (Ole Lindvall; organizer)
- Movement Disorder Society; 16th international congress of Parkinson's Disease and Movement Disorders. Dublin, Ireland (S. Fahn organizer)
- Neurostem cell meeting, Bellagio Italy (E. Cattaneo & A. Bjorklund, organizers)
- National Institutes of Health, Bethesda, MD. Stem cell lecture series (M. Boehm, organizer).
- NIMH Symposium: "Using Stem Cells for Biological and Therapeutics Discovery in Mental Illness". Bethesda, MD (D. Panchision & T. Insel; organizers)
- NINDS - 2nd IPSC Consortia Workshop. Bethesda, MD.
- Parkinson's Disease Foundation. New York, NY (R. Elliott, organizer)
- Feinstein Medical Institute for Medical Research, North Shore LI. Stem cell lecture (D. Eidelberg, organizer)
- Rutgers University - Stem cell seminar series (Z. Pang; organizer)
- NYSCF - 7th Annual Translational Stem Cell Research Conference New York, NY

- Emerging Technology Summit, New York, NY (NYC Tech Connect; organizers)
- NYSTEM - 4th Annual Meeting Keynote Lecture: New York, NY
- ISSCR - Roddenberry International Symposium on Cellular Reprogramming. San Francisco, CA (D. Srivastava & S. Yamanka; organizers).
- Symposium to honour Sir Ian Wilmut, Edinburgh, Scotland: "Driving Stem Cell Research Towards Therapy"

### **Teaching:**

- 1992 - 1993 *Teaching* practical courses in general and Neurophysiology for medical students at the University Bern, Switzerland.
- 1993 - 1996 *Mentorship* of two postdoctoral fellows and two medical students at the University of Bern Switzerland.
- 1996 - 1999 *Mentorship* of three high school students, two graduate students and two postdoctoral fellows at NINDS, Bethesda.
- 2003- curr.
- Graduate Course on Neural Development & Stem Cell Biology, Molecular, Cell & Structural Biology Program, Cornell University, 2003.
  - Organizer of monthly Developmental Biology lecture series at Sloan Kettering Institute
- 2004
- Member of Organizing Committee for Cornell/SKI Graduate School Retreat
  - Admission Committee Tri-institutional MD/PhD Program,
  - Graduate Course on Stem Cell Biology, Immunology Program, Cornell University,
  - Graduate Course on Neural Stem Cells, Molecular, Cell & Structural Biology Program, Cornell University, 2004.
- 2005
- Member of Organizing Committee for Cornell/SKI Graduate School Retreat
  - Admission Committee Tri-institutional MD/PhD Program,
  - Member Steering Committee: Tri-institutional Stem Cell Initiative (Starr Foundation)
  - Graduate Courses on Stem Cell Biology (6 hour lecture series on Adult versus Embryonic Stem Cells, Therapeutic Application of Stem Cells, and Ethical Aspects of Stem Cell Research), Molecular, Cell & Structural Biology Program, Cornell University; Neural Stem Cell lecture: Immunology Program;
- 2006
- Member of Organizing Committee for Cornell/SKI Graduate School Retreat
  - Member and President Steering Committee: Tri-institutional Stem Cell Initiative (Starr Foundation)
  - Member of the ACE committee of the Molecular, Cell & Structural Biology Program at Cornell University.
  - Graduate Courses on Stem Cell Biology (4 hour lecture series on Adult versus Embryonic Stem Cells, Therapeutic Application of Stem Cells), Molecular, Cell & Structural Biology Program, Introductory lectures for Gerstner Graduate School, SKI, and for Neuroscience Program.
- 2007
- Member and President Steering Committee: Tri-institutional Stem Cell Initiative (Starr Foundation)
  - Member of Review committee for admission to Tri-Institutional MD/PhD program.
  - Graduate Courses on Stem Cell Biology (4 hour lecture series on Adult versus Embryonic Stem Cells, Therapeutic Application of Stem Cells), Molecular, Cell & Structural Biology Program, Introductory lectures for Gerstner Graduate School, SKI, and for Neuroscience Program.
  - UNC, Chapel Hill & Duke University. Lecture series on stem cells (Lola Reid, organizer)

- 2008
- Member and President Steering Committee: Tri-institutional Stem Cell Initiative (Starr Foundation)
  - Member of Review committee for admission to Tri-Institutional MD/PhD program.
  - Graduate Courses on Stem Cell Biology: Molecular, Cell & Structural Biology Program, Introductory lectures for Gerstner Graduate School, SKI, and for Neuroscience Program; "Frontiers" lecture for MD/PhD program.
  - Organizing Committee: Tri-institutional stem cell colloquium
- 2009
- Member and President Steering Committee: Tri-institutional Stem Cell Initiative (Starr Foundation)
  - Member of Review committee for admission to Tri-Institutional MD/PhD program.
  - Graduate Courses on Stem Cell Biology: Molecular, Cell & Structural Biology Program, Introductory lectures for Gerstner Graduate School, SKI, and for Neuroscience Program; "Frontiers" lecture for MD/PhD program.
  - Organizing Committee: Tri-institutional stem cell colloquium
- 2010
- Member and President Steering Committee: Tri-institutional Stem Cell Initiative (Starr Foundation)
  - Member of Review committee for admission to Tri-Institutional MD/PhD program.
  - Graduate Courses on Stem Cell Biology: Molecular, Cell & Structural Biology Program, Introductory lectures for Gerstner Graduate School, SKI, and for Neuroscience Program; "Frontiers" lecture for MD/PhD program.
  - Organizing Committee: Tri-institutional stem cell colloquium
- 2011
- Director, Trainings program in stem cell biology at MSKCC (PI on \$ 1.8 million NYSTEM trainings grant)
  - Member and President Steering Committee: Tri-institutional Stem Cell Initiative (Starr Foundation)
  - Member of Review committee for admission to Tri-Institutional MD/PhD program.
  - Graduate Courses on Stem Cell Biology: Molecular, Cell & Structural Biology Program, Introductory lectures for Gerstner Graduate School, SKI, and for Neuroscience Program; "Frontiers" lecture for MD/PhD program.
  - Frontiers in Medicine lecture: Tri-institutional MD/PhD program
- 2012
- Director, Trainings program in stem cell biology at MSKCC (PI on \$ 1.8 million NYSTEM trainings grant)
  - Member and President Steering Committee: Tri-institutional Stem Cell Initiative (Starr Foundation)
  - Graduate Courses on Stem Cell Biology: Molecular, Cell & Structural Biology Program, Introductory lectures for Gerstner Graduate School, SKI.
  - Co-organizer: Graduate Course in Stem Cell Biology



**Special Committee Member (PhD program):**Neuroscience:

Lijian Shen,	01/01/02	4/30/04	(postdoc Rockefeller U.)
Hyojin Lee	08/01/02	3/01/07	(postdoc Burnham Institute)
Asif Maroof	07/01/05	4/7/10	
Yosif Ganat	07/01/05	7/29/10	
Elizabeth Calder	01/01/09	current	

Tri-institutional MD/PhD Program

Michael Keyoung	10/01/03	10/01/03	(resident Neurosurg. UCSF)
Hyung-Song Nam	04/15/05	3/09/09	
Kymora Bernisha Scotland	05/04/05	7/20/09	
Prabhjot Singh Dhadialla	04/15/05	3/30/09	

BCMB Program

Lihui Qian	05/25/05	10/19/09	
Xin Zhou	07/01/04	7/29/09	
Elena Fomchenko	04/01/05	6/30/10	
Zehra Dincer	07/01/05	current	
Fabien Lafaille	08/01/05	5/25/12	
Inna Lipchina	07/01/07	4/4/12	
Leszek Lisowski (examination committee)		03/10/2008	
Naira C. Rezende	02/01/08	08/02/2012	
Yuchen Qi	07/01/10	current	

Gerstner Graduate School

Shannon Yu	01/01/09	current	
Yvonne Gruber	10/01/08	current	
Justine Miller	09/01/10	current	

Columbia University

Li-Chun Cheng, Fiona Doetsch lab (Examination committee)		12/11/2008	
Gist Croft, H. Wichterle lab (Examination committee)		7/25/2011	

Foreign Universities

University of Copenhagen			
Agnete Kirkeby	12/01/2007 - 2009		

University of Surrey, UK

Bhishma Amlani (Viva Meeting)	12/01/2010		
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**Trainings Record:**Postdoctoral Fellows - **current:**

Name	Current Position	From	To	Degree	Year	Institution	Position / Fellowships
Stuart Chambers	Postdoctoral Fellow	04/01/08	current	Ph.D.	2007	Baylor College of Medicine	Postdoctoral Fellow - <b>Starr Stem Cell Scholar Fellowship</b>
Sonja Kriks	Postdoctoral Fellow	07/10/07	current	Ph.D.	2007	Salk Insitute & University of Gottingen	Postdoctoral Fellow - <b>Starr Stem Cell Scholar Fellowship</b>
Nadja Zeltner	Postdoctoral Fellow	11/01/09	current	PhD	2009	Mt. Sinai Medical School, NY	Postdoctoral Fellow - <b>Swiss National Foundation fellow</b>
Jae-Won Shim	Postdoctoral Fellow	11/01/09	current	Ph.D.	2007	Seoul National University	Research Associate - <b>NYSCF fellowship</b>
Jason Tchieu	Postdoctoral Fellow	11/15/11	current	Ph.D	2011	UCLA	Postdoctoral Fellow - <b>Starr Stem Cell Scholar Fellowship</b>
Julius Steinbeck	Postdoctoral Fellow	08/01/11	current	M.D.	2004	University of Hamburg	Postdoctoral Fellow <b>DFG - Fellowship</b>
Elsa Vera	Postdoctoral Fellow	7/11/11	current	Ph.D.	2010	Spanish National Cancer Center (CNIO)	Postdoctoral Fellow - <b>NYSTEM fellowship</b>
Vidya Prabhu	Postdoctoral Fellow	4/1/2010	current	Ph.D.	2002	University of Delaware	Senior Research Scientist
Yosif Ganat	Postdoctoral Fellow	8/1/2010	current	Ph.D.	2010	Weill Cornell - Neuroscience	Postdoctoral Fellow

## Postdoctoral Fellows - Past:

Name	Current Position	From	To	Degree	Year	Institution	Position / Fellowships
Gabsang Lee	Research Associate	07/01/08	2011	PhD	2004	Seoul National University	<b>Assistant Professor</b> , NYSCF/ Robertson Investigator
Yechiel Elkabetz	Postdoctoral Fellow	12/15/03	2009	PhD	2003	Tel Aviv University, Israel	<b>Assistant Professor</b> , Tel Aviv University, Tel Aviv, Israel
Tiziano Barberi	Research Associate	01/01/03	2006	PhD	1997	Rome State University "La Sapienza"	<b>Associate Professor &amp;</b> , ARMI, Monash University, Australia
Viviane Tabar	Assistant Attending	02/14/00	2006	MD	1989	American University Beirut	<b>Associate Attending &amp; Associate Professor</b> , MSKCC,
Anselme Perrier	Postdoctoral Fellow	03/01/02	2004	PhD	2001	University Pierre & Marie Curie, Paris , France	<b>INSERM, Group leader</b> at iSTEM, Evry-Paris, France
Christopher Fasano	Postdoctoral Fellow	06/01/07	2009	Ph.D.	2007	Albany Medical College	<b>Assistant Professor</b> , NY Neural Stem Cell Institute, Albany, NY
Mark Tomishima	Postdoctoral Fellow	03/01/02	2006	PhD	2002	Princeton University	Director SKI stem cell research facility; <b>Assistant Lab Member</b> , Sloan-Kettering
Hyesoo Kim	Postdoctoral Fellow	09/01/05	2011	PhD	2004	Seoul National University	<b>Director</b> of Johns Hopkins Stem Cell Core Facility
Noboru Sato	Postdoctoral Fellow	10/30/00	2002	PhD/M D	1996/1987	Juntendo University School of Medicine / Oita Medical University	<b>Assistant Professor</b> , Univ. of California, Riverside
Dimitris Placantonakis	Surgical Fellow (AANS research fellowship)	07/01/06	2008	M.D. PhD	2003	NYU	<b>Assistant Professor</b> , Neurosurgery NYU, New York
Sabrina Desbordes	Postdoctoral Fellow	02/01/04	2007	Ph.D.	2004	Cambridge, UK	<b>Assistant Professor</b> , Helmholtz Zentrum München Neurosurgery
George Al-Shamy	Postdoctoral Fellow	11/01/05	2007	MD	2004	American University Beirut	<b>Resident</b> , Baylor College of Medicine
Daniela Battista	Postdoctoral Fellow	12/01/09	2012	Ph.D.	2006	University of Buenos Aires, Argentina	Senior Research Scientist
David Widmer	Postdoctoral Fellow	10/01/00	2001	PhD	2000	Rutgers University	Senior Grants Officer, MSKCC
Rajasekhar Vinagolu	Senior Research Scientist	01/01/04	2010	PhD, M.Phil.	1984	Jawaharlal Nehru University, New Delhi	Senior Research Scientist

Graduate Students - **current:**

Name	Current Position	From	To	Degree	Year	Institution	Position / Fellowships
Zehra Dincer	Graduate Student	07/01/05	current	B.S.,	2001	Bogazici University, Turkey	Graduate Student
Liz Calder	Graduate Student	01/01/09	current	B.A.	2007	Colgate University	Graduate Student
Yvonne Gruber	Graduate Student	10/01/08	current	B.S.	2008	Wellsley	Graduate Student
Yuchen Qi	Graduate Student	10/01/09	current	B.S.	2007	Peking University	Graduate Student
Justine Miller	Graduate Student	03/01/11	current	B.A.	2008	Connecticut College	<b>NSF Graduate Fellowship</b>
Sarah Kishnevsky	Graduate Student	03/01/12	current	B.A.	2005	Columbia University	Graduate Student
Faranak Fattahi	Graduate Student	6/1/12	current	M.S.	2011	University of Tehran, Iran	Graduate Student

Graduate Students - **Past:**

Name	Current Position	From	To	Degree	Year	Institution	Position / Fellowships
Fabien Lafaille	Graduate Student	07/01/05	current	B.A.	2004	Hunter College	Postdoctoral Fellow, <b>Rockefeller U.</b> (J. L. Casanova lab)
Yosif Ganat	Graduate Student	11/01/05	current	B.A.	1999	Cornell, Ithaca	Postdoctoral Fellow, <b>MSKCC.</b> (L. Studer lab)
Asif Maroof	Graduate Student	11/01/05	current	M.S.	2002	Columbia University	Postdoctoral Fellow, <b>Harvard Univ.</b> (K. Eggen lab)
Inna Lipchina	Graduate Student	06/01/07	current	B.S.	2004	Brandeis University	Postdoctoral Fellow MGH, <b>Harvard Univ.</b> (K. Hochedlinger lab)
Hyojin Lee	Graduate Student	08/01/02	03/01/07	Ph.D.	2007	Weill Cornell Medical College	Postdoctoral Fellow, <b>Burnham Institute,</b> San Diego (S. Lipton)
Agnete Kirkeby	Graduate Student	09/01/07	current	M.S.	2006	University of Copenhagen, Denmark	Postdoctoral Fellow, <b>University of Lund</b> (A. Bjorklund)

**Grant Support****COMPLETED:**

01.2001.011 (Studer L.) 5/1/2001 – 4/30/2002 10%

MJ. Fox Foundation for PD \$ 80,000 / year direct costs

**Efficient Generation and Transplantation of Dopamine Neurons Derived from Cloned Mouse Embryonic Stem Cells**

“Proof of principle application of therapeutic cloning in PD mice”

Stem Cell Endowment (Studer L/Tabar V.) 1/1/2002 – 12/31/2002 2%

Hazen Foundation \$ 125,000 direct costs

**Stem Cells & Cancer Research**

“This proposal investigates the potential role of stem cells in brain tumors.”

01.2002.07 (Studer L.) 4/1/2002 – 3/28/2004 20%

MJ. Fox Foundation for PD \$ 279,228 / year direct costs (total direct: \$ 558,456)

Research and the PD Alliance

**Unlimited numbers of purified midbrain dopamine neurons from human embryonic stem cells**

“The major goal of this project is to efficiently derive, purify and fully characterize dopamine neurons from human ES cells as well as test their function in vitro and in vivo in a rat model of PD.”

1R21NS44231-01 (Studer L.) 7/1/2002 – 6/30/2004 20%

NINDS, NIH \$125,000 / year direct costs (total direct: \$ 250,000)

**Therapeutic Cloning in Parkinsonian Mice**

“This project aims at the first proof of principle application of therapeutic cloning in an animal model of PD”

Intramural Award (Studer,L/Tabar,V) 7/1/2003 – 6/30/2004 5%

MKS Society \$ 150,000 / year direct costs

**Brain Repair After Radiation Injury Using Human Embryonic Stem Cells**

“Test the application of human ES cells in repair of radiation damage to the developing cerebellum”

Research Grant (Isacson O.; Studer L: subcontract) 7/1/2002 – 6/30/2004 5%

Parkinson’s Foundation of the National Capitol Area

\$ 40,000 / year direct costs (total direct: \$ 80,000)

**The use of embryonic primate stem cells in Parkinson’s disease models**

“Study assesses the in vivo potential of pluripotent parthenogenetic monkey stem cells and their progeny in rodent and primate models of PD”

(Studer L) 1/15/2005-7/14/2005

Kinetics Foundation \$25,000 / 6 months 2%

**The generation of a GDNF expressing cynomolgus parthenogenetic monkey ES cell line**

“Derivation of GDNF expressing Cyno1-derived neural progenitors for consortium project”

- (Studer L) 4/15/2005 – 4/14/2006 2%  
 McLean Hospital, Harvard University \$40,000 / year direct costs  
**Cyno1 derived DA neurons for transplantation into Parkinsonian Primates**
- (Studer L.) 1/15/2004-1/14/2006 20%  
 Kinetics Foundation \$300,000 / year direct costs (total direct: \$ 600,000)  
**Comparison Of NIH Registered And Non-NIH Registered Human ES Cell Lines For Dopamine Neuron Derivation And Transplantation In Animal Models Of Parkinson's Disease**  
 "This study aims at defining the parameters and identifying the ES cell lines most important for future clinical implementation of human ES cell therapy in Parkinson's disease"
- 1R21NS046045-01 (Tabar V.; Studer L: Co-PI) 7/1/2003-6/30/2006 5%  
 NINDS/NIH \$125,000 / year direct costs (total direct: \$ 250,000)  
**Repair of Brain Radiation Damage via human ES cells**  
 "Assess damage to oligodendrocyte lineage after brain irradiation and use human ES cells as a source for oligodendrocyte repair"
- (Studer L) 6/15/2005-5/31/2006  
 Kinetics Foundation \$100,000 / year direct costs 10%  
**Unrestricted Grant**  
 "This study aims at providing a proof of principle demonstration of therapeutic cloning for the treatment and for in vitro disease modelling of Parkinson's disease"
- 5R01NS044819-11 (Studer L.) 4/1/2002-3/31/2007 (no cost extension)  
 NINDS, NIH \$ 321,001 / year direct costs (total direct: \$ 1,536,266)  
**Transcriptional Control of Brain Development**  
 "Investigates the role of BF1 in forebrain development"
- 1R21NS053655-01 (Studer, L.) 9/30/2005 – 9/29/2006 5%  
 NINDS, NIH 125,000 / year direct costs  
**HTS Screen for Neural Differentiation in Human ES Cells**
- RO3 ABC2 Award (Tabar V.; Studer L: mentor) 10/1/2003 – 9/30/2006 2%  
 Accelerate Brain Cancer Cure, Inc. \$ 50,000 / year direct costs (total direct: \$ 150,000)  
**Cancer Stem Cells in Human Glioma**  
 "Pursues the hypothesis that cancer stem cells may exist within primary brain tumor tissue"
- ALSA-940 (Studer, L.) 3/1/2006 – 2/28/2007 5%  
 ALS Association of America \$ 70,000  
**Human Embryonic Stem Cell derived motoneurons and skeletal muscle: In vitro applications**

Research Grant (PI: Studer, L.) 1/1/2006 – 6/30/2008

Project ALS \$ 100,000 / year direct costs (total direct: \$200,000)

**Human Embryonic Stem Cell based tools for the study of ALS**

“Project will generate BAC transgenic HB9::eGFP hESC lines and modify the lines to express mutant SOD1 gene for modelling ALS in vitro”

2006-034 (Studer, L) 10/1/2006 – 9/30/2008 8%

Starr Foundation \$ 159,180/ year direct (total direct: \$ 318,360 for LS)

**Strategies to enhance the in vivo efficacy of human ES derived dopamine neurons**

“The project will develop strategies to enhance in vivo survival of hESC derived DA neurons – particularly via introduction of intrinsic fate determinants such as Lmx1A)

2006-037 (Sander, C, Tuschl, T, Studer, L) 10/1/2006 – 9/30/2008 5% (no salary req)

Starr Foundation \$ 70,000/ year direct costs (total direct: \$ 140,000 for LS)

**The role of microRNAs in human embryonic stem cells**

“The project will characterize microRNAs specific to hESCs and perform microRNA gain and loss-of-function studies”

2006-043 (Sadelain, M.; Studer, L: subcontract) 10/1/2006 – 9/30/2008 2% (no salary req.)

Starr Foundation \$ 20,000/ year direct costs (total direct: \$ 40,000 for LS)

**Laying the foundations for integrated therapeutic cloning and globin gene transfer to treat B-thalassemia**

“The project will establish B-thalassemia hESC lines – Studer L will perform subcloning studies”

2006-013 (Rutishauser U, Studer L., Beal M.F.) 10/1/2006 – 9/30/2008 5% (no salary req.)

Starr Foundation \$ 66,918/ year direct costs (total direct: \$ 133,836 for LS)

**Improvement of ES Cell-Derived DA Neuron Grafts Using PSA-NCAM**

“The project will test the role of PSA-NCAM in host brain of ES cell graft integration”

2006-019 (Anderson S, Harrison N, Studer L) 10/1/2006 – 9/30/2008 5% (no salary req.)

Starr Foundation \$ 80,000/ year direct costs (total direct: \$ 160,000 for LS)

**Derivation of cortical GABAergic interneurons from mouse and human ES cells**

“The project will derive Lhx6+ cortical interneurons from ESCs and perform *in vitro* and *in vivo* functional analyses”

2006-026 (Mombaerts P, Studer L) 10/1/2006 – 9/30/2008 5%

Starr Foundation \$ 100,000/ year direct costs (total direct: \$ 200,000 for LS)

**Differentiation of olfactory neurons from embryonic or olfactory stem cells**

“The project will use both adult and embryonic stem cells for studies on olfactory neurogenesis”

2006-039 (Strickland S, Studer L) 10/1/2006 – 9/30/2008 5% (no salary req.)

Starr Foundation \$ 75,000/ year direct costs (total direct: \$ 150,000 for LS)

**The use of stem cells for treatment of peripheral neuropathy**

“The project is aimed at the derivation and transplantation of hESC derived neural crest”

2007-034 (Studer, L) 10/1/2007 – 9/30/2009 5%  
 Starr Foundation \$ 160,000 / year direct costs (total direct: \$ 320,000 LS)  
**Genetic and Functional Characterization of an Early hESC derived Neural Stem Cell Stage**  
 “The project is aimed at the genetic and functional characterization of early stages of hESC derived NSCs.”

2007-035 PI: Studer, L 10/1/2007 – 9/30/2009 (no salary requested)  
 Starr Foundation \$ 100,000 / year direct costs (total direct \$ 200,000 LS)  
**High throughput chemical screen in human embryonic stem cells**  
 “The project will perform HTS assays on hESCs to define compounds that regulate self-renewal and early differentiation events”

NYSTEM (PI: Studer, L.) 4/1/2008-3/31/2009 10%  
**New York State Stem Cell Grant** \$ 1,000,000 / year direct costs  
 “This is a large multi-component institutional grant to enhance stem cell research in NY state, involving supplemental funding for 7 research projects within MSKCC, educational and training funds and funds for core facility equipment and support.”

Starr Foundation (PI: Studer, L.) 9/1/2006-8/31/2010 2 %  
**SKI Stem Cell Research Core Facility** \$ 3,380,648 in total  
 “Support to establish the human ES cell core facility at SKI “SKI stem cell research facility”

1R01NS052671-01 (PI: Studer, L.) 12/1/2006-11/30/2011 10%  
 NIH/NINDS \$ 250,000 / year direct costs (total direct: \$ 1,000,000)  
**Human ES derived midbrain dopamine neurons**  
 “Strategies to enhance the survival of hESC derived DA neurons in animal models of Parkinson’s disease.”

1R01NS066390-01 (PI: Studer, L.) 7/1/2009 - 6/30/2012 12.5%  
 NIH/NINDS \$ 300,000 / year direct costs (total direct: \$ 600,000)  
**Defining fate potential in human ESC derived neural stem cells**  
 “Studies addressing the basic biology and neural fate potential of human ESC derived neural rosettes and other early neural stem-like populations”

1RC1MH089690-0110 (Multiple-PI: Anderson, S & Studer, L) 9/15/2009-8/31/2011 4%  
 NIH/NIMH \$ 137,000 / year direct costs (total direct \$ 274,000)  
**Derivation of cerebral cortical GABAergic interneurons from human iPS cells**  
 “The project will develop novel methods for GABAergic interneuron specification and isolation from human iPS cells towards exploring iPSC based models of schizophrenia”



2008-027 PI: Studer, L 10/1/2008 – 9/30/2011 10%  
 Starr Foundation \$ 160,000 / year direct costs (total direct \$ 480,000)

**Intrinsic and extrinsic fate determinants in midbrain DA neuron specification from human ESCs**

“The collaborative project will define optimized conditions for the derivation, purification and transplantation of human ESC derived midbrain DA neurons”

2008-028 PI: Studer, L 10/1/2008 – 9/30/2010 7%  
 Starr Foundation \$ 160,000 / year direct costs (total direct \$ 320,000)

**Neural Crest Specification and Modeling of Familial Dysautonomia in hESCs and hiPSCs**

“The collaborative project will define the conditions for directing hESCs into specific peripheral neuron types and address the potential of human iPSC technology to model familial dysautonomia in vitro”

2008-018 PI: Sadelain, M (Studer L Co-PI) 10/1/2008 – 9/30/2011 5%  
 Starr Foundation \$ 147,078 / year direct costs (total direct \$ 441,234)

**Improved strategies to generate human iPSC cells for disease modeling and cell therapy**

“The collaborative study will optimize the technology for human iPSC derivation including the use of non-integrating lentiviral vector technology and probe the potential of human iPSCs for disease modeling and transplantation”

2008-015 PI: Strickland, S (Studer L Co-PI) 10/1/2008 – 9/30/2011 5%  
 Starr Foundation \$ 128,000 / year direct costs (total direct \$ 384,000)

**Human embryonic stem cell-based treatment of peripheral nerve dysfunction**

“The study will develop cell therapeutic approaches in peripheral nervous dysfunction based on human ES cell derived neural crest stem cells and Schwann cell precursors”

2008-015 PI: Sander, C; (Studer L Co-PI; Tuschl L Co-PI) 10/1/2008 – 9/30/2010 2%  
 Starr Foundation \$ 86,000 / year direct costs (total direct \$ 172,000)

**The Role of miRNA in Early Neural Stem Cell Differentiation**

“The study will explore the functional role of candidate miRNAs during human ES cell differentiation towards early human neural fates”

2008-013 PI: Anderson, S.; (Studer L Co-PI; Schwartz, T Co-PI; Shi, S.H. Co-PI)  
 Starr Foundation 10/1/2008 – 9/30/2011 2%  
 \$ 97,000 / year direct costs (total direct \$ 291,000)

**Developing a cell based therapy for intractable seizures of the cerebral cortex**

“The study will explore the potential of using ES cell derived cortical interneurons for the treatment of intractable seizures in animal models of disease”

N08G-382 (Rutishauser U. :PI., Studer L: Co-PI) 1/1/2009 - 12/31/2011 10%  
 NYSTEM \$ 140,000 / year direct costs (total direct: \$420,000 – Studer lab only)

**Use of polysialic acid to improve integration of ES-derived cells into the brain**

“In this proposal we will perform gain of function studies of the PST enzyme to engineer mouse and human ES cell lines overexpressing PSA-NCAM. We provide preliminary data that this approach allows improved distribution of neural precursors and midbrain dopamine neurons in the brain of adult rodent hosts. The overarching goal is to improve ESC based grafting strategies in animal models of Parkinson’s disease.

**ACTIVE:**

C024175 NYSTEM (PI: Studer, L.) 1/1/2009-12/31/2013 5 %  
**NYSTEM – Specialized Facility** \$ 943,760 / year direct costs (total \$ 2,707,911)  
 " The SKI Stem Cell Research Facility provides key services to the stem cell community, including, a hPSC repository, teaching hPSC culture basics; directing the differentiation of pluripotent stem cells; genetic manipulation of human pluripotent stem cells."

CO26446 NYSTEM (PI: Studer)9/1/2010 - 8/31/2013 20%  
**Patient-specific human ESCs and iPSCs for Modeling Schwann Cell Differentiation and Charcot-Marie-Tooth Disease** \$ 300,000 / year direct costs (total \$ 900,000)  
 "This study will model Charcot Marie Tooth Disease in vitro using both patient specific iPSCs and PDG-hESCs. The work will also further optimize Schwann cell differentiation in pluripotent cells using a novel Sox10::eGFP BAC reporter lines generated in the Studer lab."

CO26447 NYSTEM (PI: Studer)9/1/2010 - 8/31/2013 20%  
**Modeling Pathogenesis and Treatment of Familial Dysautonomia in patient specific human induced pluripotent stem cells** \$ 300,000 / year direct costs (total \$ 900,000)  
 "This study will model clinical disease severity and specificity in FD-iPSC lines, perform rescue experiments to obtain gene corrected cells, and perform a full scale HTS assay using all currently available FDA approved drugs to identify novel candidate compounds for treating FD"

C026879 NYSTEM (PI: Studer) 7/1/2011 - 6/30/2016 5%  
**Postdoctoral Research Training Program in Stem Cell Biology** \$ 325,000 / year direct costs (total \$ 1,725,000)  
 "Allows for funding of 5 postdoc positions at Sloan-Kettering selected by peer-review and required to participate in a detailed trainings program for fellows"

C024413 NYSTEM (PI: Tabar) 1/1/2009 - 12/31/2012 5%  
**In vivo function of human iPS derived neural precursors** \$ 100,000 / year direct costs (total \$ 300,000)  
 "The award addresses human iPS cells and their potential for neural differentiation and integration in the brain using different CNS models."

22943 European Union (PI: Studer) 3/1/2010-5/31/2013 3%  
**Neurostem cell - European Consortium for Stem Cell Therapy for Neurodegenerative Diseases** \$ 35,946 / year  
 "This is large collaborative, multidisciplinary project with more than 15 laboratories in Europe. The overall goal for the consortium is to generate transplantable mesencephalic dopamine (mesDA) and striatal GABAergic neurons from pluripotent stem cell sources for cell therapy in Parkinson's and Huntington's disease respectively."

C026399 NYSTEM (PI: Chambers, Role: Mentor) 9/1/2010 - 8/31/2012 5%

**Translational control of human pluripotent cell maintenance and neural differentiation**

\$ 110,00 / year direct costs (total \$ 230,121)

"This is an awards led by senior postdoc in the lab focusing on the study of translational control during ESC differentiation from pluripotency to neural fate."

R01 NS072381 NINDS/NIH (PI: Casanova; Role: Co-PI)

9/20/2010 - 7/31/2014

5%

**Cellular dissection of herpes simplex encephalitis with iPS cells**

\$142,665 / year direct costs (total \$ 700,000)

This application uses patient specific hiPSCs to model herpes simplex encephalities in vitro using purified populations of neurons, astrocytes, oligodendrocytes and neural stem cells to model interferon response and infection and spread of HSV

U24 NS078338 NINDS/NIH (PI: Isacson: Role Project leader)

7/1/2012 - 6/30/2013

5%

**PD iPS Cell Line Consortium**

\$ 100,000 total costs

"This is a consortium grant aimed at developing cell lines, improved differentiation protocols and disease relevant assays for use in the broader stem cell community"

C024348 NYSTEM (C. Schildkraut, PI; Studer, L.: Co-PI)

1/1/2009 - 12/31/2012

6%

**Differential regulation of DNA replication during neural lineage specification in human ES cells and human iPS cells**

\$ 120,000 / year direct costs (total: \$360,000)

"The study will define the replication program of human ESCs, human iPSCs and their differentiated neural progeny using single molecule analyses"

C024414 NYSTEM (Temple, S., PI; Studer L: Co-PI)

1/1/2009 - 12/31/2011

5%

**Human RPE and induced pluripotent stem cells for Parkinson's Disease**

\$ 120,000 / year direct costs (total: \$360,000)

"This study will compare the properties of human ESC, iPSC and RPE derived dopamine neuron populations for transplantation in animal models of PD and for human disease modeling."

**Pending (selected):**

GC218627 NYSTEM (PI: Studer)

3/1/2013 - 2/28/2017

30%

**Developing a human ES cell derived dopamine neuron source for cell therapy in Parkinson's disease**

\$13,043,113 total costs

"This study is a large consortium grant with the goal of developing a human ES cell source that is suitable for clinical use with an IND target date of 2016/17."

## **Publications:**

### **I. Peer-reviewed original contributions**

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Kriks S, Shim JW, Piao J, Ganat YM, Wakeman DR, Xie Z, Carrillo-Reid L, Auyeung G, Antonacci C, Buch A, Yang L, Beal MF, Surmeier DJ, Kordower JH, Tabar V, Studer L. Dopamine neurons derived from human ES cells efficiently engraft in animal models of Parkinson's disease. **Nature**. 2011 Nov 6;480(7378):547-51 doi: 10.1038/nature10648. [Epub ahead of print]

A Poised Chromatin Platform for TGF- $\beta$  Access to Master Regulators. Xi Q, Wang Z, Zaromytidou AI, Zhang XH, Chow-Tsang LF, Liu JX, Kim H, Barlas A, Manova-Todorova K, Kaartinen V, Studer L, Mark W, Patel DJ, Massagué J. **Cell**. 2011 Dec 23;147(7):1511-24.

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