

CURRICULUM VITAE  
CYNTHIA A. RASK, M.D.

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CURRENT POSITION

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[Independent Consultant](#)

April 1, 2006 – current

*Responsibilities and Accomplishments*

- Consulted half-time (approximately 20 hours per week) for a gene therapy biotechnology company located in San Diego in the capacity of Vice President of Clinical Development and Regulatory Affairs, assisting the company in their interactions with FDA, designing and initiating Phase 2 clinical studies of their two lead products, as well as helping them to identify and map out the regulatory strategies for other products that are still in preclinical development
- Consulted approximately half-time for several medium to large CROs, providing neurologic and regulatory expertise to their clients' clinical development programs
- Served as Interim Chief Medical Officer for Satoris, Inc., a diagnostics company developing a protein array plasma diagnostic test for early stage Alzheimer's disease, mild cognitive impairment (MCI) and other neurologic diseases
- Consulted on approximately a half-time basis

PREVIOUS POSITIONS

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[Vice President, Clinical Development, Institute for OneWorld Health \(iOWH\)](#)

July 2005 – March 31, 2006

*Responsibilities and Accomplishments:*

- Manage clinical development team at iOWH (in absence of a Medical Director and Director of Clinical Operations)
- Manage staff and consultants writing the clinical study report for a phase 3 study of paromomycin in the treatment of visceral leishmaniasis (VL) – the largest Phase 3 trial ever conducted in Bihar, India
- Design Phase 2/3 study to determine optimum dose of paromomycin
- Design phase 4 study to evaluate utilization of paromomycin in rural India
- Write and edit parts of the Common Technical Document for an NDA submission to the Indian Government for paromomycin to treat VL
- Leader of the neurotoxicology subteam of the artemisinin malaria project
- Made presentations on behalf of iOWH to the Bill and Melinda Gates Foundation, Neurotoxicology Advisory Board, and collaborators at Walter Reed Army Institute of Research
- Clinical advisor to the Diarrhea Project Team
- Recruited and hired staff for the clinical group

Director, Division of Clinical Evaluation and Pharmacology/Toxicology; Office of Cellular, Tissue and Gene Therapies; Center for Biologic Research and Evaluation (CBER); FDA  
October 2002 – July 2005

*Responsibilities and Accomplishments:*

Regulatory, regulatory policy, scientific and management responsibilities, which included:

- Supervised all clinical and pharmacology/toxicology reviews for all cell, tissue, and gene therapies (for all clinical indications including oncology, neurology, cardiovascular, autoimmune and metabolic diseases) under the 900+ INDs and IDEs within the Office
- Supervised all letter comments to IND sponsors from reviewers in the Clinical and Toxicology Branches:
  - advice and information letters,
  - clinical hold and remove clinical hold letters,
  - advice and concurrence or non-concurrence on Special Protocol Assessments for Phase 3 studies
  - concurrence or non-concurrence of requests for Fast Track Designation
  - minutes from pre- Phase 3 and other sponsor meetings
- Developed and managed the Division's budget
- Developed FDA policy in consultation with the Office and Center Directors and the Deputy FDA commissioner
- Oversaw formulation and writing of FDA guidance documents
- Presented FDA policy to external constituent groups
- Presented Office policy to the Center Director, the FDA Commissioner and/or members of Congress
- Made educational presentations to groups within and outside of FDA
- Recruited and supervised the Clinical Branch Chief and Toxicology Branch Chief
- Recruited six new Medical Officers, an Oncology Team Leader, a General Medicine Team Leader and three new Pharmacology/Toxicology reviewers
- Mentored novice clinical reviewers
- Met all congressionally mandated PDUFA and MDUFMA timelines without exception
- Various additional responsibilities, including many of those listed below under the description of roles and responsibilities for a Medical Officer

Acting Deputy Director, Office of Cellular, Tissue and Gene Therapies, CBER, FDA  
January 1999 – October 2002

*Responsibilities and Accomplishments:*

Regulatory, regulatory policy, scientific and management responsibilities for the Office as a whole, assisting the Acting Office Director, while continuing to serve as Director of DCEPT (temporary detail while national search conducted for a permanent Office Director).

- Assisted the Office Director in meeting review and policy goals and timelines
- Administered Office budget

Medical Review Officer, Division of Clinical Trial Design and Analysis, CBER, FDA  
January 1999 – October 2002

*Responsibilities and Accomplishments:*

- Primary medical/clinical reviewer of 30 - 40 new INDs per year (many for neurological indications, but including a variety of diseases, including Type I diabetes mellitus, peripheral arterial disease, coronary artery disease, and metabolic storage diseases). Product classes reviewed included cytokines, therapeutic proteins, monoclonal antibodies, cellular implantation (including xenotransplantation), tissue transplantation, and gene therapy.
- Clinical review of several Biologics License Applications (BLAs) and BLA supplements
  - Clinical review of beta interferon 1- $\alpha$  (Rebif™) for treatment of relapsing and remitting multiple sclerosis, and documented first ever FDA decision to break orphan exclusivity on the basis of demonstrating clinical superiority to previous orphan product (reviews available on FDA website)
  - Clinical review for an additional indication for an interferon product (review available on FDA website)
- Participated in multiple pre-IND meetings
- Drafted advice and clinical hold letters to IND sponsors
- Reviewed new clinical protocols submitted to support Phases 1, 2 and 3 of clinical development and Phase 4 for post-marketing studies
- Provided comments for End of Phase 1, End of Phase 2 and pre-BLA meetings with sponsors
- Primary medical and neurologic reviewer for trials of botulinum toxins for the treatment of headache, spasticity and hemifacial spasm for the Office of Vaccines Research and Review (OVRR)
- Provided neurologic consultations on blood products for the Office of Blood Research and Review (OBRR), as well as to other Branches within our Division and Office and to the Center for Devices and Radiologic Health (CDRH)
- Member for more than 4 years and co-chair for one year of a CBER - NINDS working group providing input to the Translational Research program at National Institute of Neurologic Diseases and Stroke (NINDS)
- Helped organize the Biologic Modifiers Response Advisory Committee (BMRAC) meeting on July 13-14, 2000 on the use of stem cells in the treatment of neurological disorders
- Member of FDA's Xenotransplantation Working Group
- Member of the Long-Term Monitoring in Gene Transfer Trials Working Group
- Member of the InterCenter Diabetes Working Group
- Member of the Gene Therapy Clinical Trials Working Group (with OBA and RAC at NIH)
- FDA representative to several patient advocacy groups, including the Neuronal Ceroid Lipofuscinosis Research Alliance, Project ALS, and the Foundation Fighting Blindness
- Gave presentations on behalf of FDA at scientific meetings, including ASGT, ISCT, BIO and others
- Government representative to the Board of Directors of the American Society for Experimental Neurotherapeutics (ASENT)

FDA AWARDS

1999: "For exemplary performance in taking the lead neurology reviewer role for INDs"

2000: "For significant, exceptional performance and teamwork in planning and executing a public workshop on issues related to regulation of cellular therapies in neurological disorders"

2001: "For exceptional performance in review and management of responses to an Agency request for information regarding Gene Therapy clinical trials"

2002-2003: For outstanding leadership in the development of the Division and continued application of high clinical standards to regulatory submissions"

2003: "For exceptional dedication and contribution in quickly responding to the SCID adverse event reported to the agency"

2004: "For outstanding effort, foresight, and leadership in organizing a highly collaborative advisory committee meeting on allogeneic islets as a therapy for severe type -1 diabetes"

2004: "For exceptional insight and teamwork in planning and executing a highly successful public advisory committee meeting on issues relating to insertional mutagenesis by retroviral vectors for gene therapy"

2005: "For exceptional foresight and customer service in identifying and acting on an emerging policy issue to facilitate review and regulation of a novel combination technology"

Senior Clinical Scientist (Senior Medical Director), Medical Affairs, Genentech, Inc.  
1996 – December 1998

Clinical Scientist (Medical Director), Medical Affairs, Genentech, Inc.  
December 1993 –1996

*Responsibilities and Accomplishments:*

- Directed Genentech's clinical neuroscience program, designing and overseeing the conduct of the recombinant human nerve growth factor (rhNGF) clinical studies in diabetic and HIV-associated peripheral neuropathies (Phases 1 through 3)
- Neuroscience advisor to the departments of Neuroscience Research, Business Development, Marketing and other functional areas within Genentech
- Scientific reviewer on committees within Genentech including the Research Review Committee and the Clinical Research Review Committee
- Member of preclinical-clinical team to evaluate neuroscience development projects
- International Clinical Team Leader and clinical science representative to the International Project Team for the Phase 3 program for diabetic neuropathy
- Lead the clinical team in preparation for a BLA filing for rhNGF in HIV-associated sensory neuropathy
- Made presentations to the outside medical community and to investors about the clinical neuroscience program

**Associate Director of Clinical Research, Abbott Laboratories**

September 1991– December 1993

*Responsibilities and Accomplishments:*

- Oversight of the clinical development of tiagabine (Gabitril®), which resulted in NDA submission and approval
  - Designed and wrote protocols (Phases 1, 2 and 3) and study reports
  - Medical monitor for phase 1, 2, and 3 clinical studies
  - Managed phase 1, 2, and 3 clinical studies that enrolled approximately 1500 patients
  - Managed a clinical team comprised of 10 clinical research associates, two clinical project managers, one physician and three secretaries
  - Primary liaison to study investigators and opinion leaders specializing in epilepsy
  - Coordinated the activities of statisticians, data managers, pharmacologists, drug formulation and packaging representatives, quality assurance, regulatory agency representatives, and several contract research organizations
  - Co-monitored study sites with clinical research associates
  - Wrote publications and presentations for scientific meetings
- Participated in the filing of an IND for an antipsychotic agent
- Participated in meetings with consultants and opinion leaders specializing in neurological and psychiatric diseases

**EDUCATION**

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A.B., Cornell University	1976
M.D., University of Minnesota	1980

**TRAINING**

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Intern and Assistant Resident in Medicine, University of Rochester Associated Hospitals Program	1980-1982
Assistant Resident in Neurology, University of Rochester	1982-1984
Chief Resident in Neurology, University of Rochester	1984-1985
Research Fellow, Minnesota Comprehensive Epilepsy Program	1985-1989
Clinical Epilepsy Fellow	Dr. Robert J. Gumnit, Supervisor
Neuropharmacology Fellow	Dr. Ilo E. Leppik, Supervisor
EEG Fellow	Dr. Fernando Torres, Supervisor

**BOARD CERTIFICATION**

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National Board of Medical Examiners	1981, Certificate Number 232293
American Board of Neurology and Psychiatry	1988, Certificate Number 31015
American Board of Clinical Neurophysiology	1989, Qualified in EEG and Evoked Potentials

**LICENSURE**

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Minnesota	License Number 29247
Wisconsin	License Number 29888 (inactive)
New Hampshire	License Number 8223 (inactive)

New York

License Number 147316 (inactive)

ACADEMIC APPOINTMENTS

University of Minnesota, Instructor in Neurology	1985-1988
University of Minnesota, Assistant Professor of Neurology	1988-1989
Dartmouth Medical School, Assistant Professor of Neurology	1989-1991

PROFESSIONAL ORGANIZATIONS

American College of Physicians (Associate Member)	1981-1986
American Academy of Neurology (Junior and Active Member)	1982-present
American Epilepsy Society (Active Member)	1985-present
American EEG Society (Member)	1985-1991
Eastern EEG Society	1985-1991
American Society for Experimental Neurotherapeutics (ASENT)	1999-present
Board of Directors for ASENT	2002-present
American Society of Tropical Medicine and Hygiene	2005-present

AWARDS

Merritt-Putnam Fellowship, Epilepsy Foundation of America	1986-1987
Wilder-Penfield Fellowship, Epilepsy Foundation of America	1987-1988

RESEARCH PROJECTS

## Antiepileptic Drug Research

“Safety and Efficacy of Depakote Monotherapy in the Treatment of Complex Partial Seizures: A Plasma Concentration-Response Study,” Principal Investigator.

“A Multi-Center, Open Label, Long-Term Evaluation of the Safety of Felbamate in Subjects with Epilepsy,” Principal Investigator.

“A Single and Multiple Dose Pharmacokinetics Study of C1-945 in Epileptic Patients Maintained on Phenytoin,” Co-Investigator.

“A Double-Blind, Placebo-Controlled, Multicenter Study to Determine the Safety and Efficacy of Gabapentin as Add-on Therapy in the Treatment of Partial Seizures,” Co-Investigator.

“Evaluation of Phenytoin levels After IM and IV ACC-9653 Administration in Epileptic Patients on Chronic Oral Dilantin Monotherapy (Phenytoin Prodrug),” Co-Investigator.

“An Open-Label Add-On Study of MK-801 in Patients with Frequent Seizures,” Co-Investigator.

“Phase II Multicenter Controlled Clinical Trial of ADD03055 (Wallace Laboratories) in Patients with Partial Seizures,” Co-Investigator.

“A Study to Determine the Efficacy and Safety of Zonisamide (CI-912) in the Treatment of Partial Seizures in Medically Refractory Patients,” Co-Investigator.

## Catamenial Epilepsy Research

A Clinical Study of Catamenial Epilepsy conducted while an epilepsy fellowship at the University of Minnesota Comprehensive Epilepsy Program (MINCEP)

Effects of Estradiol and Progesterone Replacement on Seizure Threshold in Ovariectomized DBA/2J and E1 Mice (Two Genetic Models of Epilepsy)

## SELECTED PRESENTATIONS

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“Clinical Trial Design,” The Regulatory Compliance and Human Subjects Protections Branch of the National Institute of Allergy and Infectious Diseases and the Food and Drug Administration, April 15, 2005, Bethesda, MD

“How Can CBER/FDA Facilitate Industry Progress Through Clinical Development?” Facilitate Cell & Gene Therapy Forum 2005, January 24, 2005, Washington, D.C.

“Regulatory Strategies for Developing New Cellular and Gene Therapies for Neurologic Indications,” Funding Opportunities and Regulatory Steps for Developing Biological Therapeutics in Neuroscience symposium, Society for Neuroscience Annual Meeting, October 25, 2004, San Diego, CA

“Spinal Cord Injury Therapeutics - Regulatory Challenges,” Institute of Medicine Committee on Spinal Cord Injury: Strategies in a Search for a Cure, September 27, 2004 - September 28, 2004, Washington, D.C.

“Clinical Studies in Spinal Cord Injury: Regulatory Guidelines,” International Clinical Trials Workshop on Spinal Cord Injury, February 20–21, 2004, Vancouver, British Columbia

Retinal Gene Therapy: Clinical Issues for Diseases with Orphan Classification,” Foundation Fighting Blindness, September 29, 2003, Gaithersburg, MD

“Introduction to the IND Process: How to Develop a Gene Transfer Product” workshop at the American Society for Gene Therapy Annual Meeting, March 13-14, 2003, Arlington, VA.

“Clinical Trials of Nerve Growth Factor for Neuropathy,” NGF 98: The 5<sup>th</sup> NGF Conference, May 29 – June 2 1998, Stockholm Sweden, (Special guest: Nobel Laureate, Dr. Rita Levi-Montalcini)

## PUBLICATIONS

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**Rask CA, Hamill RW.** Neurology in aging and long-term care. Seminars in Neurology. 1985; 5: (1) 57-64.

**Rask CA, Hamill RW.** Burden of neurologic disease in long-term care and aging (abstract). Neurology 1985; 35 (S1): 301.

**Rask CA, Leppik IE, Loewenson RB, Neugebauer R.** Occurrence of catamenial epilepsy (abstract). Neurology 1987; 37 (S1): 144.

Leppik IE, Marienau KJ, **Rask** CA, Loewenson RB, Jacobs MJ, Beniak TE. A short neuropsychological test battery for detecting subclinical impairment from antiepileptic drugs (abstract). Epilepsia 1987; 28 (5): 613.

Leppik IE, Anhut H, **Rask** CA, Schatzie B, Graves N, Strump G, Thomann P. Gabapentin kinetics may not be altered by chronic phenytoin therapy (abstract). Presented at the 17<sup>th</sup> Epilepsy International Congress.

Leppik IE, Marienau KM, Graves NM, **Rask** CA. MK-801 for epilepsy: a pilot study (abstract). Neurology 1988; 38 (S1): 405.

Leppik IE, **Rask** CA, Watridge C, Graves NM, Murthy VS, Boucher BR, Wilder BJ, Turlapaty P. Pharmacokinetics of phenytoin prodrug after IV and IM administration (abstract). Neurology 1988; 38 (S1): 186.

**Rask** CA, Gates JR, Torres F, Maxwell RE. Subdural electrodes, somatosensory evoked potentials, and direct cortical stimulation in epilepsy surgery (abstract). Epilepsia. 1988; 29 (5): 685.

Talwar D, **Rask** CA, Torres D. Clinical manifestations in patients with occipital spikewave paroxysms (abstract). Epilepsia 1988;29 (5): 665.

Leppik IE, **Rask** CA. Pharmacokinetics of antiepileptic drugs during pregnancy. In: Neurological problems in pregnancy. Seminars in Neurology 1988; 8 (3): 240-246.

Leppik IE, **Rask** CA, Graves NM, Holmes GB, Slavin M, Anhut H, Schmidt B. Pharmacokinetics of gabapentin in patients treated with phenytoin. Pharmacotherapy 1989; 9(3): 196.

Leppik IE, Boucher R, Wilder BJ, Murthy VS, **Rask** CA, Watridge C, Graves NM, Rangel RJ, Turlapaty P. Phenytoin prodrug: preclinical and clinical studies. Epilepsia 1989; 30 (S2): S22-S26.

Leppik IE, Boucher BA, Wilder BJ, Murthy VS, Watridge C, Graves NM, Rangel RJ, **Rask** CA, Turlapaty P. Pharmacokinetics and safety of a phenytoin prodrug given IV or IM in patients. Neurology 1990; 40:456-460.

Richens A, Chadwick D, Duncan J, Dam M, Morrow J, Gram L, Mengel H, Shu V, Pierce M, **Rask** CA, Hightower B. Safety and efficacy of tiagabine HCl as adjunctive treatment for complex partial seizures. (abstract) Epilepsia 1992; 33 Suppl 3:119.

Talwar D, **Rask** CA, Torres F, Clinical manifestations in children with occipital spikewave paroxysms. Epilepsia 1992; 33 (4): 667-674.

During M, Mattson R, Scheyer R, **Rask** CA, Pierce M, Mckelvy J, Thomas V. The effect of tiagabine HCl on extracellular GABA levels in the human hippocampus. (abstract) Epilepsia 1992; 33 Suppl 3:83.

Hom AC, Leppik IE, **Rask** CA. Effects of estradiol and progesterone on seizure sensitivity in oophorectomized DBA/2J Mice and C57/EL hybrid mice. Neurology 1993; 43:198-204.

Hefti F, Gao WQ, Nikolics K, Rosenthal A, Shelton D, Phillips HS, Treanor JS, Widmer HR, **Rask** CA, Burton EL, Winslow JW. Role of neurotrophic factors and their receptors. In "Life and death in the nervous system." Eds: Ibanez, CF, et al, Elsevier Science, Stockholm, 1995.

**Rask** CA, Apfel S, Adornato B, Cornblath D, Kessler J, Petty B, Chaudhry V, Schwartz S, Dyck PJ, Burton LE. An overview of the clinical experience with systemic administration of nerve growth factor in treating peripheral nervous system disorders. (abstract) Annals of Neurology 1995; 38:317.

Dyck PJ, Peroutka S, **Rask** CA, Burton LE, Baker M, Lehman K, Gillen DA, Hokanson J, O'Brien PC. Intra-dermal recombinant human nerve growth factor induces pressure allodynia and lowered heat-pain threshold in humans. Neurology 1997; 48:501-505.

**Rask** CA and Escandon E. Neurotrophin treatment of peripheral sensory neuropathies. In "Handbook of experimental Pharmacology," Neurotrophic Factors. Ed: Hefti, F. 1998; 134:53-79.

Apfel SA, Kessler JA, Adornato BT, Litchy WJ, Scanders C, **Rask** CA, the NGF Study Group. A randomized double-blind, placebo-controlled trial of recombinant human nerve growth factor in symptomatic diabetic polyneuropathy with small fiber sensory loss. Neurology 1998; 51:695-702.

**Rask** CA, Carlsen RC, Elias KA. Site of action of recombinant human nerve growth factor in diabetic neuropathy appears to be small sensory neurons in the peripheral nervous system: phase II data support preclinical results. (abstract presented at the 17<sup>th</sup> Joint Meeting of the British Endocrine Societies, 1998).

Elias KA, **Rask** CA. Accumulating evidence suggests recombinant human nerve growth factor (rhNGF) may specifically affect small sensory neurons in the peripheral nervous system. (abstract presented at the Neurodiab meeting, 1998).

**Rask** CA, Adornato BT, Sanders C. Clinically relevant doses of recombinant human nerve growth factor (rhNGF) have a large margin of safety. (abstract presented at the Endo '98 meeting).

**Rask** CA, Sanders, C, Hauessler JH. Positive results of phase II recombinant human nerve growth factor (rhNGF) trial triggers two phase III trials to confirm efficacy and safety in diabetic neuropathy. (abstract presented at the annual meeting of the European Neurological Society, 1998).

**Rask** CA. The biological actions of nerve growth factor (NGF) in the peripheral nervous system. European Neurology (supplement), 1998.

McArthur JC, Yiannoutsos C, Simpson DM, Adornato BT, Singer EJ, Hollander H, Marra C, Rubin M, Cohen BA, Tucker T, Navia BA, Schifitto G, Katzenstein D, **Rask** C, Zaborski L, Smith ME, Shriver S, Millar L, Clifford DB. A phase 2 trial of nerve growth factor for sensory neuropathy associated with HIV infection. AIDS Clinical Trials Group Team 291. Neurology 2000; 54(5): 1080-1088.

Dyck PJ, Turner DW, Davies JL, O'Brien PC, Dyck JB, **Rask CA**. Electronic Case-Report Forms of Symptoms and Impairments of Peripheral Neuropathy. Canadian Journal of Neurological Sciences 2002; 29: 258-266.