

NIH on Funding Clinical Trials

Scott Janis, Ph.D.
NINDS Clinical Trials Group



NIH FY 2004 Clinical Trials by Institute

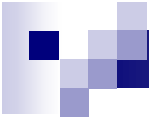
IC	FY 2004 Actual Obligations	IC Rank based on Total Budget	FY 2002 Clinical Trials	IC Rank based on CT Dollars	Clinical Trials as % of IC Budget	IC Rank based on CT as % of Budget
NCI	4,739,255	1	799,955	1	17%	3
NHLBI	2,878,691	3	244,982	3	9%	10
NIDCR	383,282	17	20,320	15	5%	15
NIDDK	1,821,803	5	177,000	5	10%	7
NINDS	1,501,207	6	98,964	8	7%	13
NIAID	4,155,447	2	694,655	2	17%	4
NIGMS	1,904,838	4	0	20	0%	20
NICHD	1,242,361	8	191,405	4	15%	5
NEI	653,052	12	58,969	11	9%	8
NIEHS	632,392	13	2,325	18	0%	19
NIA	1,024,754	10	76,085	10	7%	12
NIAMS	501,066	14	32,536	13	6%	14
NIDCD	382,053	18	3,201	17	1%	18
NIMH	1,381,774	7	119,187	7	9%	9
NIDA	990,953	11	171,170	6	17%	2
NIAAA	428,669	16	50,851	12	12%	6
NINR	134,724	23	1,630	19	1%	17
NHGRI	479,073	15	8,645	16	2%	16
NIBIB	287,129	21	0	20	0%	20
NCRR	1,179,058	9	94,792	9	8%	11
NCCAM	116,978	24	30,499	14	26%	1
NCMHD	191,471	22	0	20	0%	20

(dollars in thousands)

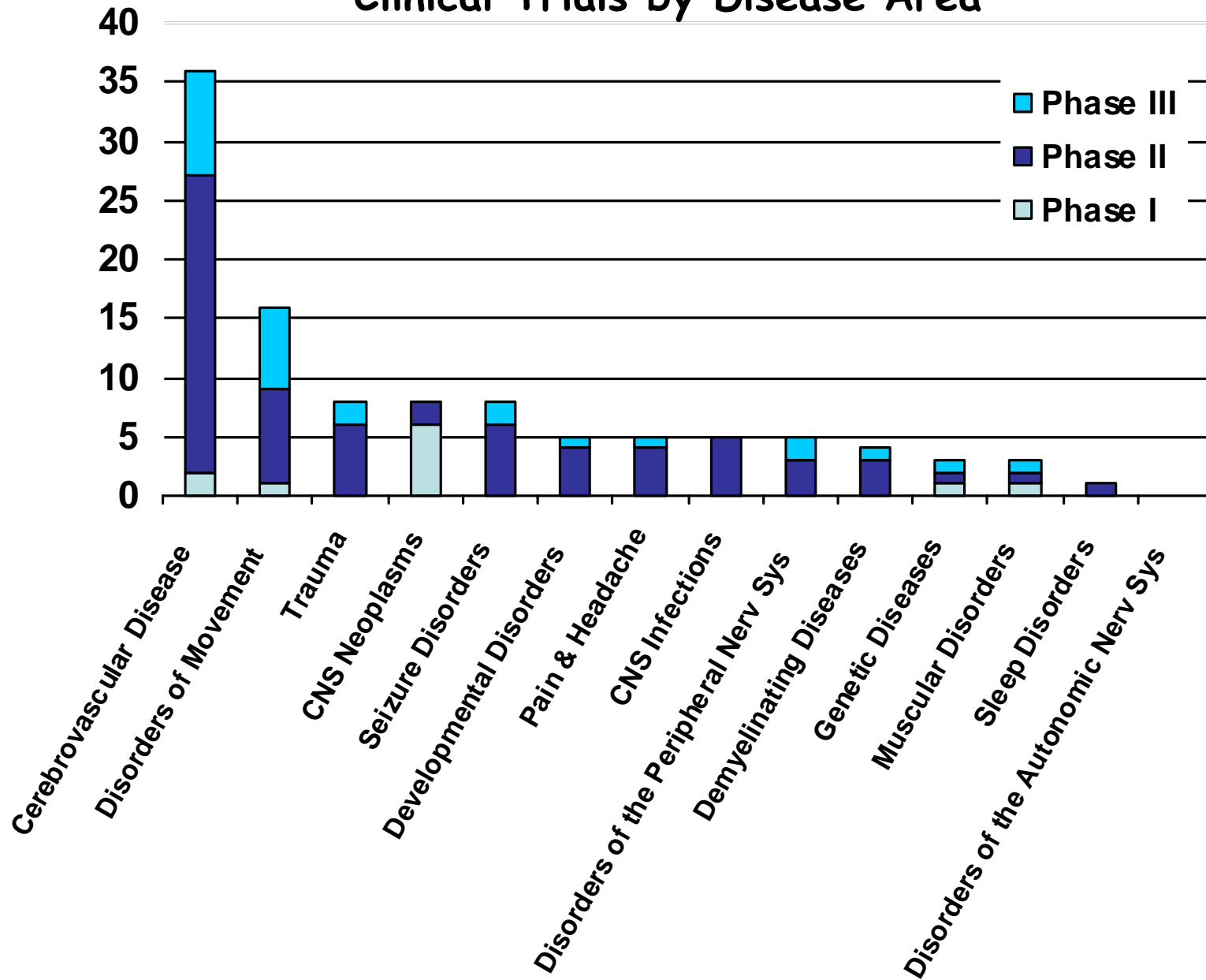


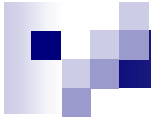
NINDS Clinical Research

- FY 2004 NINDS spent \$1.19B on 3,667 individual grants of this amount
 - \$372M (31%) was spent on 773 (21%) individual grants require human subjects approval from an IRG
 - of these grants, there are 115 phase I, I/II, II, or Phase III clinical trials
 - In addition, 1282 other types of clinical studies
- 2004 NINDS ranks 4th in number of ongoing clinical trials and 8th overall in NIH expenditure on clinical trials (~100M)
- NINDS oversees approximately 60 DSMB's, in addition, staff are involved in SMC and IMM monitoring



Clinical Trials by Disease Area





Pre-Clinical

} R01 Basic Research

Safety and Dose

} R01 Pilot Clinical Trials

Dose Confirmation

Phase III Protocol

Manual of Operations

} R34 Planning Grant

Perform Phase III Trial

} R01 Randomized Trial



Pilot Study Grant - Overview

- Purpose: to provide specific data about an intervention necessary to design a subsequent efficacy trial.
- Mechanism: R01 through PAR -03-174.
- Limit: 3 years and generally < \$350K/yr.
- Review: IC review



Pilot Study - Examples

- Studies of safety and tolerance, PK, or activity (e.g., markers of therapeutic activity).
- Studies to optimize the intervention strategy (e.g., optimal dose, duration).
- Studies to select the best of several possible interventions or dosages, based on tolerance or markers of activity.
- Studies to define the target population.



Pilot Study

- We are not interested in funding:

- Pilot studies designed to test for efficacy in a small study
- Pilot studies designed to test the feasibility of doing the trial: e.g.:
 - What is the optimal outcome measure to use?
 - Will data collection run smoothly?
 - Can clinicians correctly administer the intervention?
 - How should we recruit study subjects?
- Pilot studies designed to determine the power for a subsequent phase III trial



Planning Grant

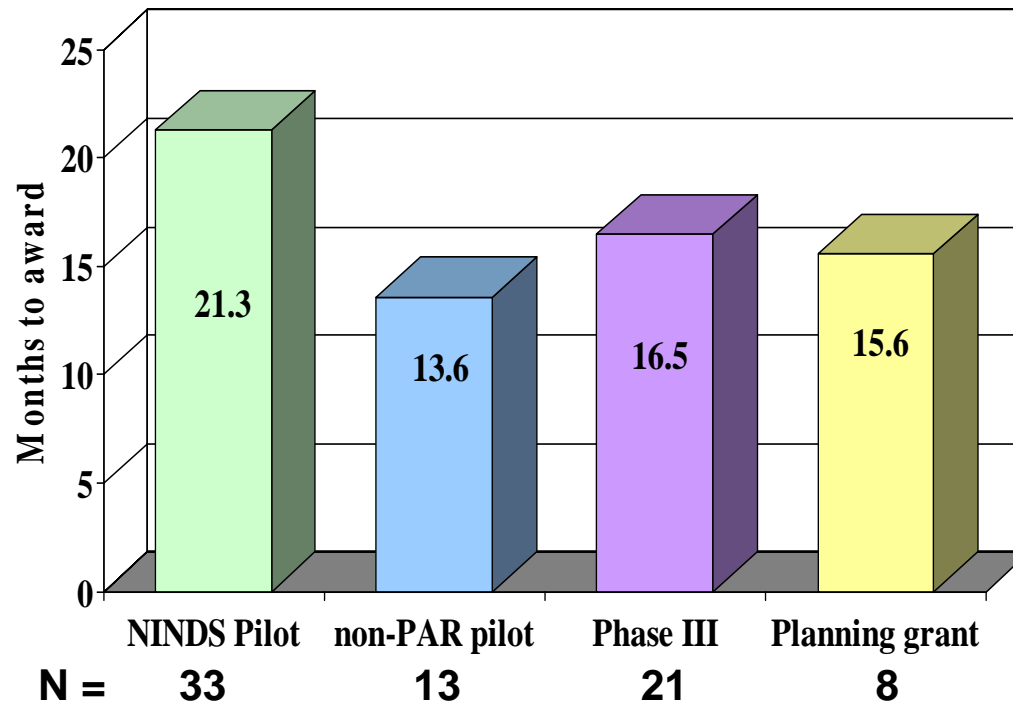
- Purpose: to finalize the study protocol, to develop a detailed manual of operations and set of data collection forms, and to organize a group of clinical investigators.
- Limit: 12 mos. and \$150,000 (total).
- Mechanism: R34 through PA -03-054.
- Review: IC review.



Efficacy Trial Grant

- Purpose: to compare the efficacy of a new treatment intervention in humans relative to a control.
- Mechanism: R01 and U01.
- Review: IC review.

Avg. months to Award = 17.9



	Max	Min
NINDS PAR Pilot	35.9	10.6
Non-PAR Pilot	20.7	7.9
Phase III	37.4	9.6
Planning Grant	23.3	9.9



Future trends & Challenges



Clinical Research in Neurology

- Thousands of clinical projects
 - Collecting genetic and other tissue samples
 - Large national epidemiological projects
 - Studies of new methods in radiology
 - Studies of brain organization and behavior



Goals for Clinical Trials in Neurology

- Design larger, simpler trials that enroll more patients (i.e., simplify exclusion criteria and data collection)
- Test treatment effects that are both clinically relevant and realistic
- Enlist support of many neurologists, especially those who are in practice on their own—go beyond the usual academic centers that participate in neurological trials



Goals for Clinical Trials in Neurology

- Everyone will follow clinical trial results when everyone participates in the trials
- Make participating in trials as common for neurologists as it is for cardiologists and oncologists.
- We can make trials more friendly by making the forms very simple and using the same forms over and over in different trials.
- As funding becomes scarce we must insure that good, quality, scientifically sound trials are the result that will bring new therapies to the patients.



Clinical Research Resources

NINDS Clinical Research Web-site

<http://www.ninds.nih.gov/clinicalresearch>