Designing a Disease-Specific Master Protocol

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Ellen V. Sigal, PhD

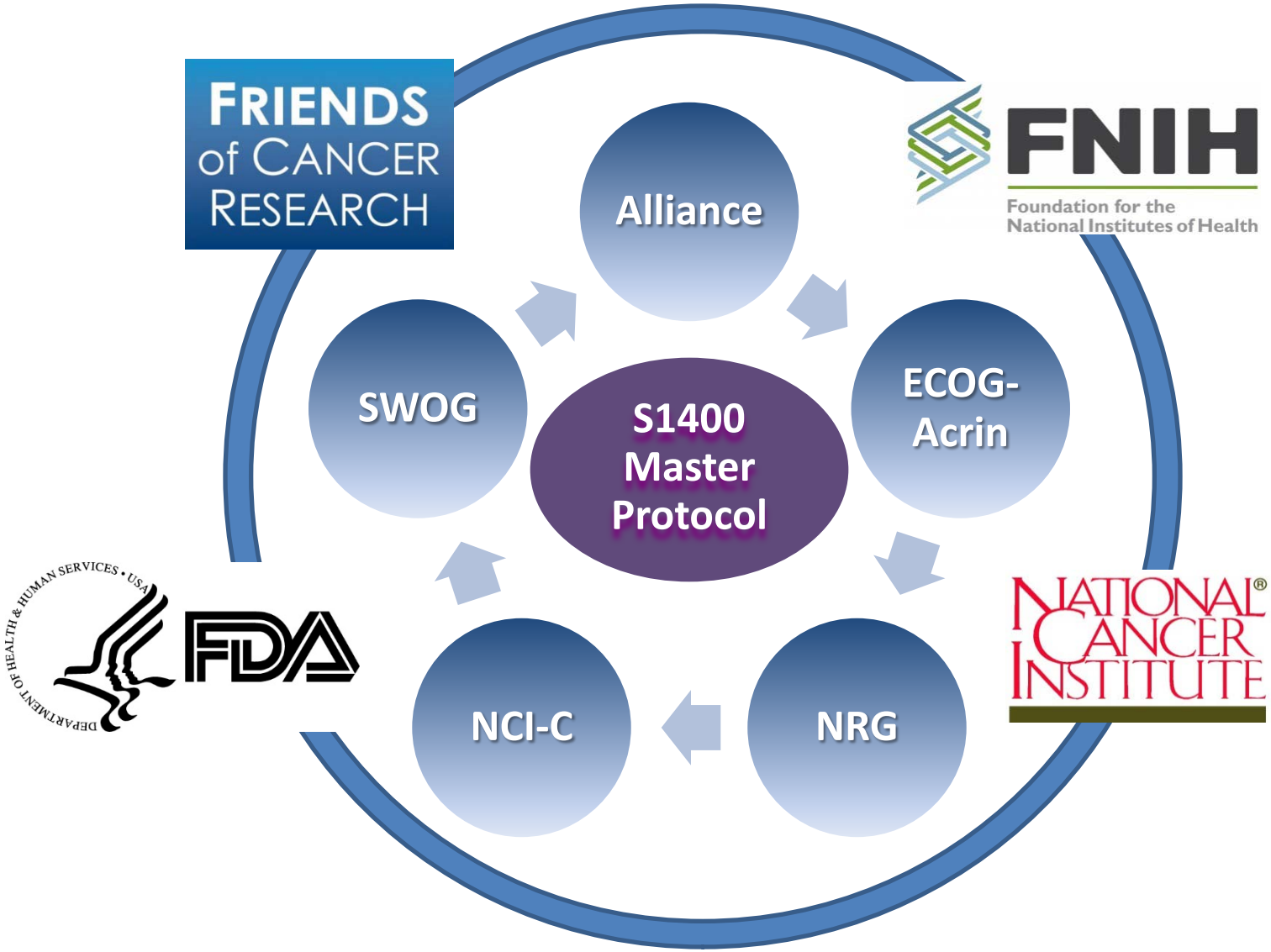
Chair & Founder, Friends of Cancer Research

FRIENDS of CANCER RESEARCH

Trial Barriers

- Potential new therapies are often tested independently from other therapies, despite seeking to treat the same condition
- Every new trial, requires protocol review by numerous oversight entities
  - new phase III trials require an average of 36 administrative or regulatory approvals and averages more than 2 years
- Approximately 4% of adult cancer patients enroll in clinical trials
  - inability to meet accrual goals is a frequent factor causing trial closure, wasting time, money, and limited patient resources
- Molecularly-targeted therapies may be present in only small fractions of the patient population making accrual impossible

S1400 Master Protocol
Unique Private-Public Partnerships with the NCTN



Major Elements

- **Setting:** S1400 2nd-Line Therapy of Squamous Non-Small Cell Lung Cancer
  - Phase II/III Biomarker-Driven Master Protocol
  - Multi-arm randomized, controlled master registration protocol
- **Agents:** Candidate drugs must demonstrate activity against a measurable target with a proposed predictive biomarker
- **Screening:** Molecule-specific tests to be developed as a companion diagnostics

Benefits of Master Protocols

- **Efficiency:** Single protocol can be amended as needed as drugs enter and exit the study
- **Patient Access:** A single trial should reduce the screen failure rate and minimize patient rejection
- **Consistency:** Each drug tested identically
- **Cost:** Shared resources can save wasteful spending
- **Predictability:** If efficacy and safety criteria are met, the drug and companion diagnostic will be approved
- **Speed:** Bringing safe and effective drugs to patients sooner than they might otherwise be available

Lessons Learned

1. **Collaboration** and extensive communication
2. **Accrual** – this is about patients
3. **Science** – right platforms, endpoints and drugs are key
4. **Accountability** and strong governance
5. **Flexibility**

Expert Working Group

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Ellen Sigal

FNIH Foundation for the National Institutes of Health

David Wholley

SCHOOL OF MEDICINE
Division of Medical Oncology
UNIVERSITY OF COLORADO ANSCHUTZ MEDICAL

Fred Hirsch

UCDAVIS HEALTH SYSTEM

David Gandara

FRED HUTCH
40 YEARS OF CURES 1975–2015

Mary Redman

THE UNIVERSITY OF TEXAS
MD Anderson Cancer Center
Making Cancer History®

Vali Papadimitrakopoulou

NATIONAL CANCER INSTITUTE

Jeff Abrams
Shakun Malik
Jack Welch

Yale CANCER CENTER

Roy Herbst

AMGEN

David Chang

MERCK
Be well

Eric Rubin

Lilly

Richard Gaynor