

Non-NIH Funding

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Officer Johnson runs into one of those "gray areas" of the law.

“I shall not today attempt further to define the kinds of material I understand to be embraced within that shorthand description; and perhaps I could never succeed in intelligibly doing so. But I know it when I see it...”

**Justice Potter Stewart
Jacobellis v. Ohio
378 U.S. 184 (1964)**

Perspective (RCR)

- Chair, Cystic Fibrosis Foundation/CFFTI Clinical Research Committee.
- Member, AHA Renal/Lung Study Sections
- Member of NIH/NIDDK/CSR Special Emphasis and DSMB Panels
- Ad hoc reviewer for MRC (UK) and US-Israel Binational Science Foundation
- Former IRB Chair
- Consultant to OHRP and FDA regarding Pediatric Research

- Applicant--NIH, CFF, AHA, NKF, HHMI, DOD, SPAR, BWT

Commonalities of NIH and Foundation Grant Reviews

- Clearly stated Hypothesis
- Clearly articulated Specific Aims to test the Hypothesis
- Sense that the topic is **important** and **worthy of further investigation**
- Sense that the proposal is **“doable”**

- **Differences between NIH and Foundations often result from how highlighted terms are defined**

Contrasting Agendas

NIH

- Public Health and Welfare
- Training and developing future academic leaders

Foundations

- Health and welfare of a specific group or area of interest
- Training of physicians and researchers in that area
- Luring experienced investigators into new areas
- Fostering collaboration

Contrasting Criteria

NIH

- **Significance**
- **Approach**
 - Including Human/Animal Subjects
- **Innovation**
- **Investigators**
- **Environment**
- **Overall Evaluation**
- **(Budget)**

CFF (RRT and CRC)

- **Critique**
- **Relevance**
- **Investigator(s)**
- **Adequacy of Effort**
- **Resources and Environment**
- **Ethical Issues and Institutional Commitment**
- **Budget**
- **Overall Recommendation**

Significance vs. Relevance

- **Significance (NIH)**

“Does this study address an important problem? If the aims of the application are achieved, how will scientific knowledge or clinical practice be advanced? What will be the effect of these studies on the concepts, methods, technologies, treatments, services, or preventative interventions that drive this field?”

Specific focus from RFA's and PAR's

- **Relevance (CFF)**

“Does this application address an important question that is relevant to CF Clinical research or clinical management?”

AKA “Need to know *versus* nice to know”

- **Other Foundations**

- AHA
 - Cardiovascular Disease or Stroke Related
- NKF
 - HTN, Dialysis, Bone, Dyslipidemia, Nutrition
- DOD
 - Area driven/RFA like
- SPAR
 - Asthma

Approach/Innovation vs. Critique

- **Approach (NIH)**
Are the conceptual or clinical framework, design, methods, and analyses adequately developed, well-integrated, well-reasoned, and appropriate to the aims of the project? **Does the applicant acknowledge potential problem areas and consider alternative tactics?**
- **Innovation**
Is the project original and innovative? For example: Does the project challenge existing paradigms or clinical practice; address an innovative hypothesis or critical barrier to progress in the field? Does the project develop or employ novel concepts, approaches or methodologies, tools, or technologies for this area?
- **Critique (CFF)**
 - Discuss and evaluate the scientific merit of the research design, approaches and methodology. Are the hypotheses/aims logical? Is the approach valid and adequate? Are the procedures feasible? **Are potential difficulties and/or limitations adequately discussed?** **Will the research produce new data or confirm existing hypotheses?** What is the significance of the proposed study relative to the state of the science? **Will it provide information required to develop a larger study?** **How can the study be improved?**

Investigators

- **NIH**

Are the investigators appropriately trained and well suited to carry out this work? Is the work proposed appropriate to the experience level of the principal investigator and other researchers? Does the investigative team bring complementary and integrated expertise to the project (if applicable)?

- **CFF**

Discuss and evaluate the background, experience and qualifications of the applicant and key investigators.

- Discuss and evaluate the time commitments of the investigator(s) and other collaborative personnel relative to conducting and/or supervising the study.

Environment

- **NIH**

Does the scientific environment in which the work will be done contribute to the probability of success? Do the proposed studies benefit from unique features of the scientific environment, or subject populations, or employ useful collaborative arrangements? Is there evidence of institutional support?

Animal and Human Subjects

- **CFF**

- Discuss and evaluate the facilities, resources and equipment at the disposal of the investigator(s). Pay particular attention to the availability of CF cells and/or patients.
- Discuss the inclusion of letters of support, human subject approval, release forms and other institutional needs for this project. If the proposal places human subjects at risk, are the risks reasonable relative to the expected benefits?

Budget

- **NIH**

Summary recommendation by study section

(ie adequacy or reduction of modules)

- **CFF**

- Evaluate the budget relative to the research plan. Identify any items in each of the budget years that should be deleted or adjusted and provide the basis for this recommendation.

- Budgets are smaller, with smaller indirects
- Focus on minimal essentials to accomplish project.
- Equipment requests scrutinized carefully.

Overall Evaluation/Recommendation

- **NIH**

Criteria and Impact Score
(1-9)

Percentile

“Pay Line”

- **CFF**

Scientific merit (1-9)

CF Relevance (1-9)

Will sometimes partially fund
interesting parts if whole
proposal not funded

“Information derived from such
studies will hopefully lead to
submission to other funding
agencies, such as the
National Institutes of Health
(NIH).”

What constitutes "Doability" or Feasibility?

• NIH

- Preliminary data (and publications)
- Track record and productivity
- Expertise
- Resources
- Fishing expeditions used to be **STRONGLY** discouraged, but have gained some acceptance as "non-biased approaches"

• CFF

- **Can often have less preliminary data**
- Track record important
- **Commitment to CF**
- **Expertise, especially if not applied to CF in the past.**
- Resources
- Fishing expeditions may be okay, as long as they are fishing in a high quality pond

Special Areas

- **Clinical Research**
- **Training and Junior Faculty Awards**
 - traineeships, fellowships and other awards
"to cultivate the next generation of
experienced CF physicians and scientists"
- **Pilot and Feasibility**
- **CFF/NIH Approved but Unfunded**

Clinical Research (CFF)

- Clearly stated hypothesis, experimental design or methods, and discussion of key problems and confounders, potential pitfalls and proper controls.
- Definite evidence by the P.I. of the leadership qualities required to bring together other scientists to investigate, in an interdisciplinary fashion, areas clearly related to CF.
 - Competency and depth as independent investigator
 - Evidence of his/her intention and ability to carry out clinical research that is related to CF for several years
- Funding priority for projects developing therapeutic interventions to interrupt the abnormal pathophysiology of CF.
- Study design must include a fully documented power analysis justifying sample size and also include a biostatistician in the professional personnel.
- Active association with a CFF Care Center; clinical trial applications must originate from an accredited CFF Care Center.
- Scored for Scientific Merit (1-9) and Perceived Impact (1-9)

Training Awards (CFF)

Post-doctoral Fellowships

Labs of established
researchers

Preference to recent
graduates

Commitment to CF-related
research

Clinical Fellowship

Specialized training to
prepare candidates for
careers in academic
medicine.

CF-related research and
career goals

- Review heavily emphasizes sponsorship and educational plan.
- For clinical fellowships, a personal statement demonstrating commitment to CF is heavily weighed

Junior Faculty Awards (CFF)

Physician Scientist

Newly trained pediatricians and internists to enhance clinical proficiency in CF-related sub-specialties and to develop research capabilities. Designed for professionals with a commitment to CF research and care to develop into independent biomedical investigators.

Clinician Scientist

Clinically trained physicians with a commitment to research to develop into independent biomedical research investigators in CF related areas

Completed postdoctoral training defined an area of research interest and sponsor(s) to provide research guidance

- Review heavily emphasizes sponsorship and educational plan.
 - Coursework
 - Techniques
 - Track record of sponsor and institution
 - Feeling that an independent research program will emerge
- A personal statement demonstrating commitment to CF is heavily weighed

Pilot and Feasibility (CFF)

Develop and test new hypotheses and/or new methods
Develop and test hypotheses or methods new to CF
research

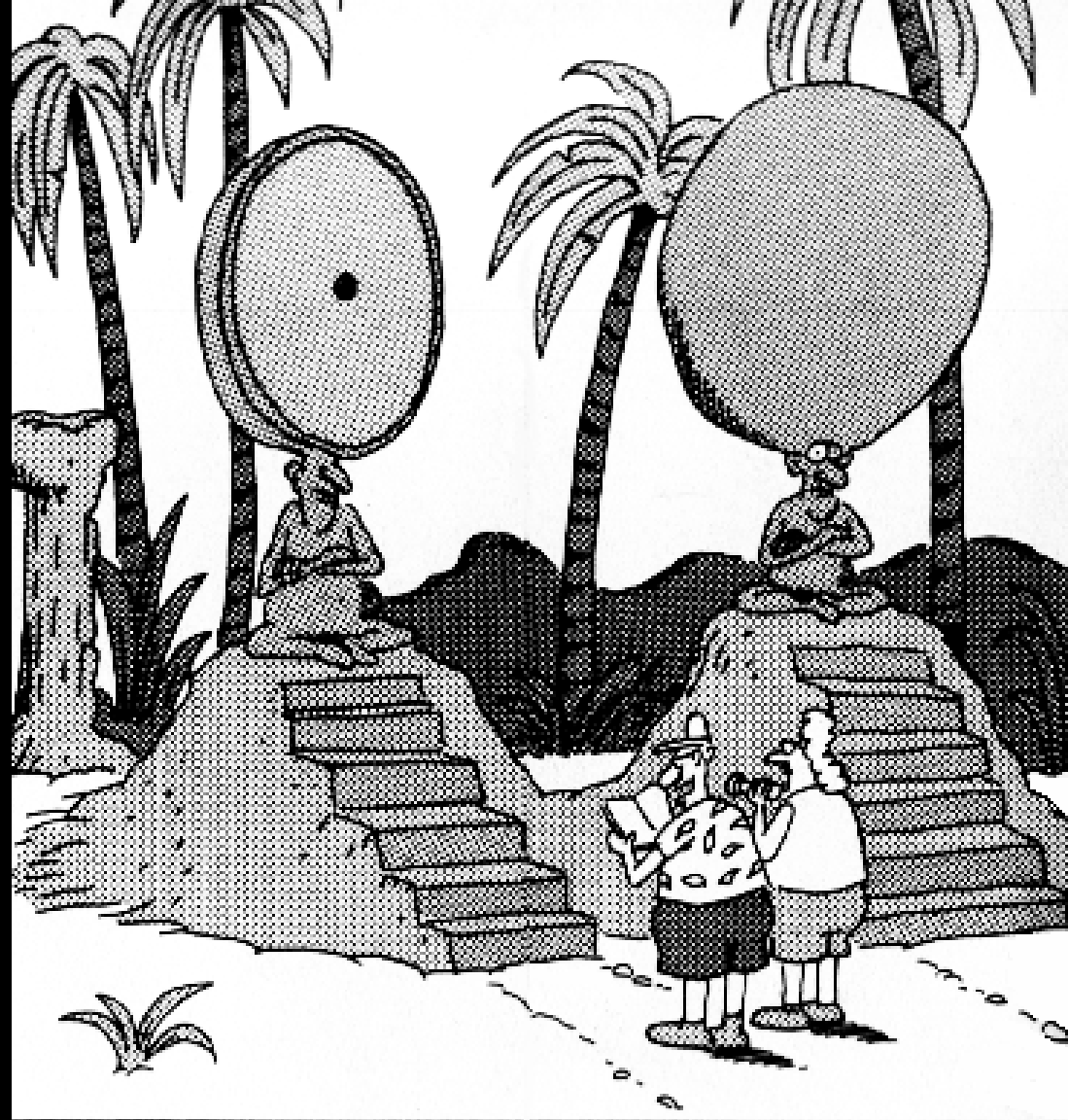
Also, targeted to scientists starting their careers as independent researchers.

- Preference given to candidates with at least two years relevant postdoctoral experience and a faculty appointment, who have not yet achieved the rank of associate professor or its equivalent.

The intent of this award is to enable investigators to collect sufficient data to compete successfully for support from the NIH or other funding agencies

Conclusions

- Private Foundations have more limited budgets and focus
- Priorities are set and more tightly defined
- Want to draw committed people into their fields
- Provide opportunities for support of training and young faculty
- Major goal is to support research efforts in their field to make such research competitive in review at the NIH level, as well as to support niche efforts that may not be competitive at such a level.



“Well, this guidebook is worthless! It just says these people worshipped two gods: one who was all-knowing and one who was all-seeing — but they don’t tell you which is which, for crying out loud!”