

**Day one-presentation-session IV**  
**David Goldstein**

**Patient Participation with No Advocacy Group: Web-based Recruitment and Screening**

**David S. Goldstein, MD, PhD**

*Clinical Neurocardiology Section, CNP/DIR/NINDS/NIH, Bethesda, MD*

In rare and common neurodegenerative diseases, by the time the patient develops symptoms, neuronal loss is already advanced. People with pre-symptomatic disease offer great hope for understanding pathogenetic mechanisms and testing potential neuroprotective agents, but how can such people be found? Patient recruitment via support or advocacy groups is expensive and time consuming. A novel alternative approach is web-based recruitment and screening. Potential advantages include extremely wide catchment, cost efficiency, and sharing of potential participant rosters. Data entries can themselves provide valuable research information. An example of such a website is that developed for the intramural NINDS protocol, "Biomarkers of Risk of Parkinson Disease," In conjunction with placement of a Google Ad. After about 8 months the site has already been visited by more than 13,000 people. Almost 750 people have entered their risk factor data yielding 84 with multiple risk factors rendering them eligible for further study, without involvement of an advocacy group.



## *Uniting Rare Diseases*

### *Advancing Rare Disease Research: The Intersection of Patient Registries, Biospecimen Repositories and Clinical Data*

#### *Session IV*

#### *Patient Participation & Outreach Activities/Patient Advocacy*

***David Goldstein M.D., Ph.D***

*Clinical Neurocardiology Section, CNP/DIR/NINDS/NIH*

### *Participation of Patients with No Advocacy Group*



# Challenges



- **In rare and common neurodegenerative diseases, by the time the patient develops symptoms, neuronal loss is already advanced.**
- **Support or advocacy groups emphasize searches for “cures” of symptomatic disease.**
- **People with pre-symptomatic disease offer great hope for understanding pathogenetic mechanisms and testing potential neuroprotective agents, but how can such people be found?**
- **Patient recruitment is expensive and time consuming.**
- **Rosters of potential research participants are not shared among investigator groups.**

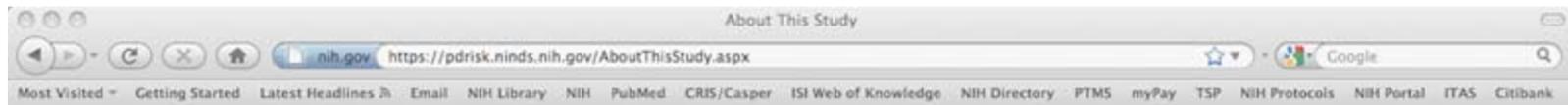
# Potential Advantages of Web-based Recruitment

---



- **Extremely wide catchment including by people with early disease or with statistical risk factors.**
- **Cost efficiency.**
- **Rosters of potential research participants can be shared.**
- **Data entries can themselves provide valuable research information.**

# Example: Biomarkers of Risk of Parkinson Disease



## About This Study

Welcome!

There are two main purposes of this study.

About Us

The first purpose is to determine whether people who have characteristics that may be risk factors for Parkinson disease (PD) have objective evidence—"biomarkers"—that the disease process is actually going on. The evidence we are looking for is loss of nerves and nerve cells that use particular chemicals, called catecholamines. PD patients typically have a loss of nerves and nerve cells that use the catecholamines dopamine and norepinephrine as chemical messengers.

About This Study

Register Here

The second purpose is to determine whether people who have abnormal "biomarkers" actually develop PD during several years of follow-up.

Privacy Act Statement

PD Risk Information

PD Risk Details

Click Here to Continue →

Continue

Info. Summary

Confidential Website

Contact Us

Exit





# Registration



## Register Here

Welcome!

About Us

About This Study

Register Here

Privacy Act Statement

PD Risk Information

PD Risk Details

Info. Summary

Confidential Website

Contact Us

Exit

If you wish to register for our study, "Biomarkers of Risk of Parkinson Disease," please enter your e-mail address below and click the SEND button.

We will immediately reply to the e-mail address you give, to confirm that you are at that e-mail address. Then we can proceed.

and   
Please type in your e-mail address      Your state of residence  
Then Click Here →

If you are returning to this website from a previous visit, please enter your ID number  and click here:  If you don't remember your ID number, type in your e-mail address and State of residence above, and we'll e-mail your ID number to you.



# Consent



## Privacy Act Notification Statement

Welcome!

About Us

About This Study

Register Here

**Privacy Act Statement**

PD Risk Information

PD Risk Details

Info. Summary

Confidential Website

Contact Us

Exit

Collection of this information is authorized under 42 U.S.C. 241, 242, 248, 281, 282, 284, 285a, 285b, 285c, 285d, 285e, 285f, 285g, 285h, 285i, 285j, 285l, 285m, 285n, 285o, 285p, 285q, 287, 287b, 287c, 289a, 289c, and 44 U.S.C. 3101. The primary use of this information is to enable you to register online for a clinical research protocol at the National Institute of Neurological Disorders and Stroke (NINDS), National Institutes of Health (NIH) to further study the risk of individuals developing Parkinson Disease. This information may be disclosed to NIH researchers for research purposes only and to NIH staff and contractors responsible for maintaining this website. Submission of this information is voluntary; however, in order for us to register you online for the NIH Clinical Research Protocol, "Biomarkers of Risk of Parkinson Disease," you should complete all the fields.

There are **no risks** to your providing information to us about you. **You may discontinue your participation at any time**, for any reason.

If based on the information you provide we decide that you do not qualify for further evaluation, you will be informed within 2 weeks by e-mail that you do not qualify. **We may retain the information you provide** for research purposes only.

If we decide that you do qualify for further evaluation, you will be informed within 2 weeks by e-mail that you qualify, and we will direct you to a secure web-site where you can enter personal identifying information, and then we will send you by mail a cover letter and the consent form for the study. If you qualify, and you do not choose to provide this information, you will not be considered further, and we may retain the information you provided at the non-secure web-site, for research purposes only.

I have read, understand, and agree  Agree  Disagree →





# Research Data



- [Patient Summary Report Long Page](#)
- [Patient Summary Report](#)
- [PI Evaluation Form](#)
- [PI Reply Record](#)
- [Patient Eligibility Report](#)
- [Response Status Report](#)

[Log Out](#)

## Risk Factor Summary

Total responses:	747
Total with risk factors:	647
Total with 4 risk factors:	23
Total with 3 risk factors:	86
Total with 2 risk factors:	181
Total with 1 risk factor:	357
Total with 0 risk factors:	100
Total with genetic risk:	477
Total with olfactory risk:	187
Total with orthostatic hypotension risk:	179
Total with REM behavior disorder risk:	226
Total eligible:	84
Total not eligible:	663



# Administrative Database



PD Risk Admin Page

http://pdriskadmin.ninds.nih.gov/Admin/PatientSummaryReport.aspx

Most Visited Getting Started Latest Headlines Email NIH Library NIH PubMed CRIS/Casper ISI Web of Knowledge NIH Directory PTMS myPay TSP NIH Protocols NIH Portal ITAS Citibank

[Patient Summary Report Long Page](#)
[Patient Summary Report](#)
[PI Evaluation Form](#)
[PI Reply Record](#)
[Patient Eligibility Report](#)
[Response Status Report](#)
[Log Out](#)

### Patient Summary Report

Total: 744 [Export to Excel](#)

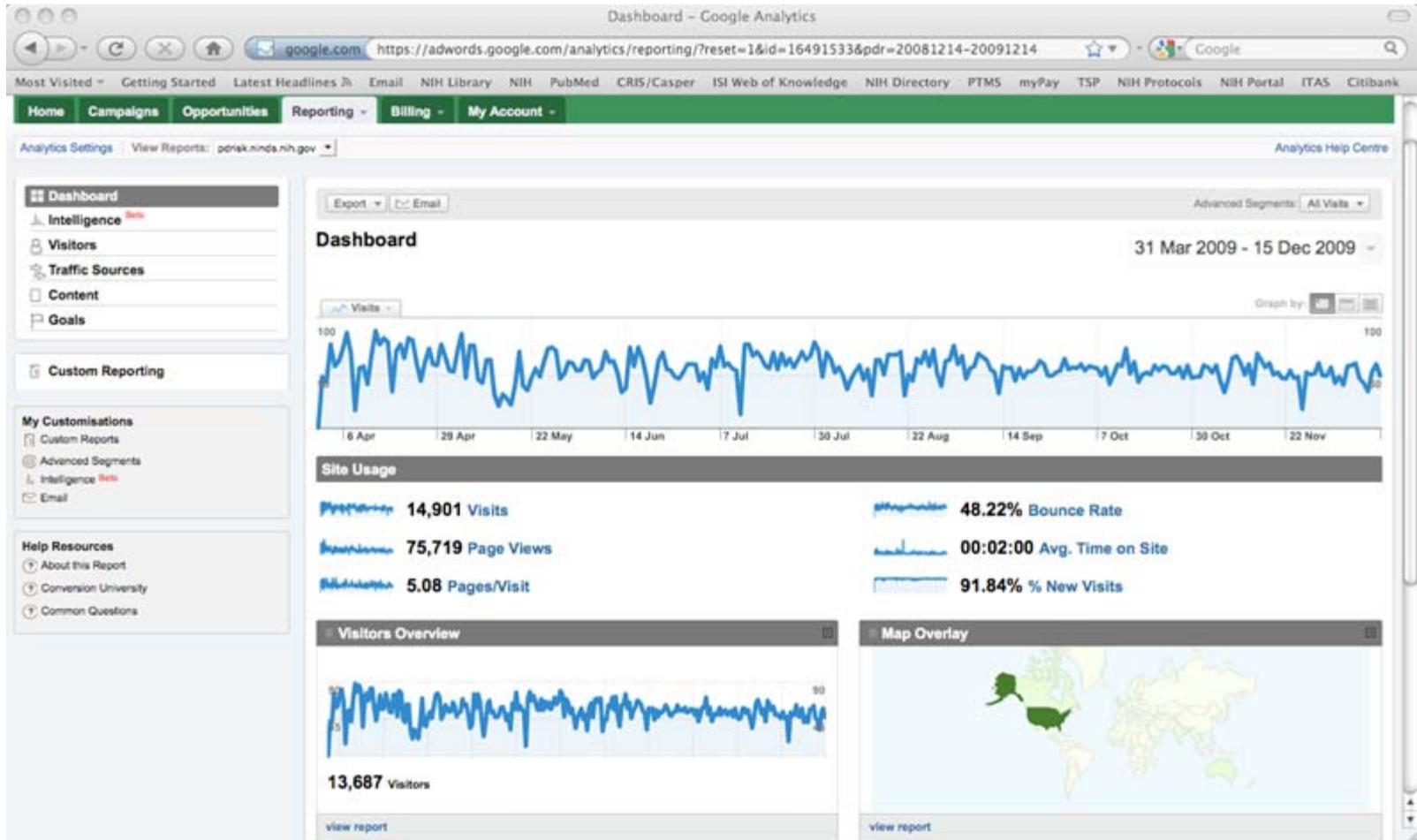
1 2 3 4 5 6 7 8 9 10 ...

UID	SubmitDate	Name	DOB	Gender	Address	City	State	zip	Phone	Cell	Email	Family Genetic	Decreased Smell	Orthostatic Hypotension	Sleep Disorder	Eligible?	Replied Date	Consent Date	Risk Detail	PI Notes
1	101360	12/15/2009										Yes	No	No	No	No	12/15/2009		<a href="#">Detail</a>	<a href="#">PI Notes</a>
2	101357	12/14/2009										No	No	No	No	No	12/15/2009		<a href="#">Detail</a>	<a href="#">PI Notes</a>
3	101355	12/14/2009										No	No	No	No	No	12/15/2009		<a href="#">Detail</a>	<a href="#">PI Notes</a>
4	101354	12/14/2009										No	No	No	No	No	12/15/2009		<a href="#">Detail</a>	<a href="#">PI Notes</a>
5	101353	12/13/2009										Yes	No	No	No	No	12/15/2009		<a href="#">Detail</a>	<a href="#">PI Notes</a>
6	101350	12/12/2009										No	No	No	Yes	No	12/15/2009		<a href="#">Detail</a>	<a href="#">PI Notes</a>
7	101349	12/12/2009										Yes	Yes	No	Yes	Yes	12/15/2009		<a href="#">Detail</a>	<a href="#">PI Notes</a>
8	101347	12/12/2009										No	No	Yes	No	No	12/15/2009		<a href="#">Detail</a>	<a href="#">PI Notes</a>
9	101346	12/11/2009										No	Yes	No	Yes	No	12/15/2009		<a href="#">Detail</a>	<a href="#">PI Notes</a>
10	101343	12/11/2009										Yes	No	No	No	No	12/11/2009		<a href="#">Detail</a>	<a href="#">PI Notes</a>

1 2 3 4 5 6 7 8 9 10 ...



# Wide Catchment





## Conclusions

---

- A protocol-specific website is a powerful, cost-effective tool for informing the public, recruiting potential research subjects, obtaining research data, constructing a subject database, and tracking study status.
- Such a website also offers unique opportunities for collaboration and data sharing.