

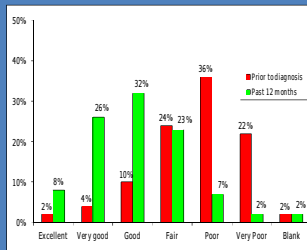


Importance of Data Collection

**IPOPI Biennial Meeting
October 31, 2014**

Presented By:
Marcia Boyle
Immune Deficiency Foundation





Survey Research

Better Information for Better Outcomes

More than 35 surveys of patients and medical professionals since 1995

Highlights include:

- National Patient Surveys (1996, 2002, 2007, 2012)
- National Treatment Surveys (1997, 2003, 2008, 2013)
- Prevalence Survey (2005)
- National Internet Treatment Survey of PIDD in the U.S. (2010)
- Medicare Access to Care Surveys: Patients, Physicians, Hospital Pharmacist Directors (2006, 2007)
- Physician Surveys
 - Pediatrician Survey (2007)
 - Family Practitioner Survey (2009)
 - Use of IVIG in Treatment of PI – IDF and AAAAI (2009)
 - Pulmonologist Survey (2011)
- Early vs. Delayed Diagnosis of SCID (2010)
- Primary Immunodeficiency & Women's Reproductive Health Survey (2012)

Patients in the General Community: A National Probability Survey of the Prevalence of Patients diagnosed with PI

IDF Prevalence Survey

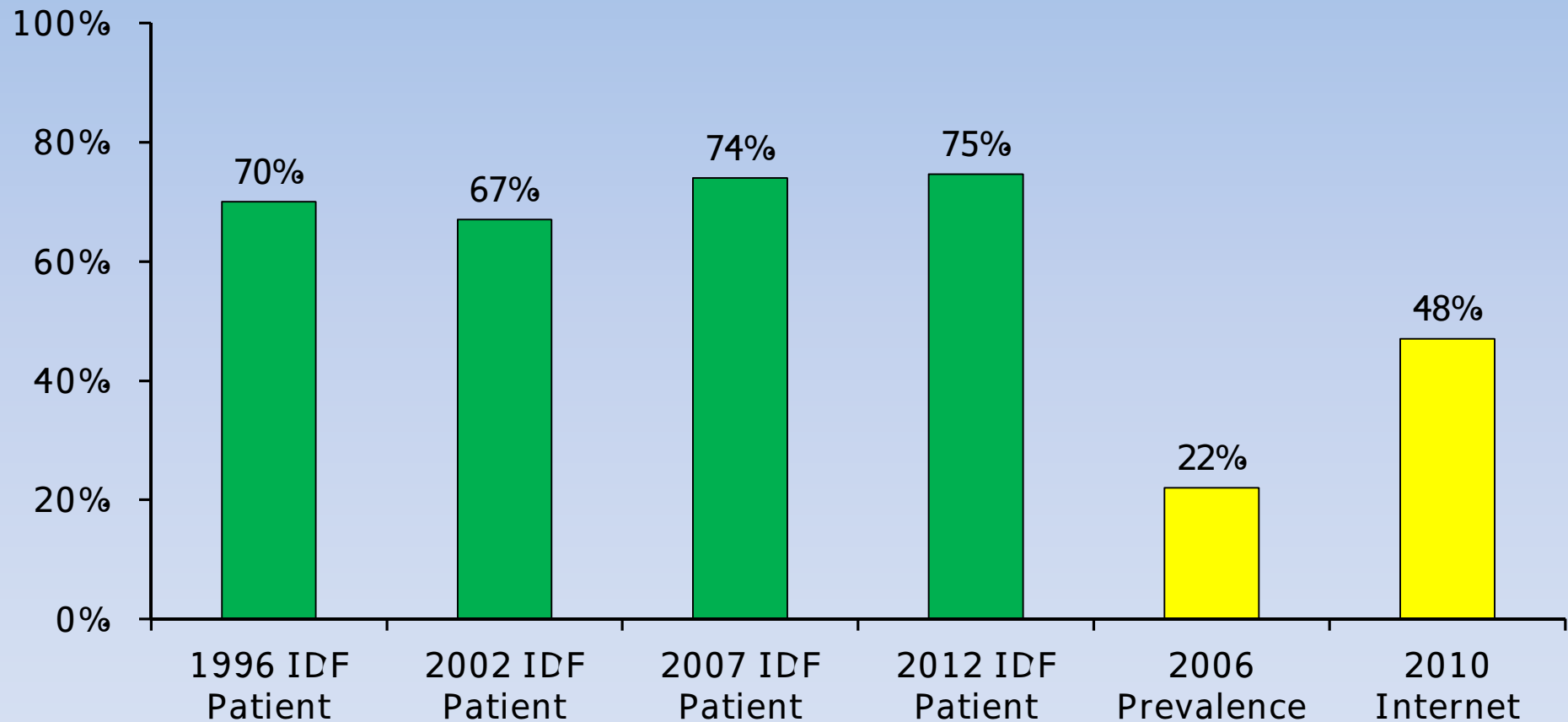
- National telephone survey of 10,000 households
- 1 in 1,200 persons diagnosed with PI
- Estimate of 250,000 PI in the U.S.
- 95% confidence level around the estimate suggests 152,000 to 361,000 diagnosed PI in the U.S.



IDF: Patient Surveys Since 1995

- Mail-based, those patients who are known to IDF
 - National Patient Surveys (1995, 2002, 2007, 2012)
 - Overall look at ALL diagnoses
 - Unique sample, those who have come to IDF since last survey or have not participated in a prior patient survey
 - National Treatment Surveys (1996, 2003, 2008)
 - Look at those who currently receive Ig therapy
 - Sample is a random selection of all known IG users from IDF database
 - Detailed examination of impact of Ig therapy on patients, and their experiences, challenges and opinions with Ig therapy issues.
 - Over 1,000 completed surveys for ALL
 - Although nationally distributed, non-probability sample

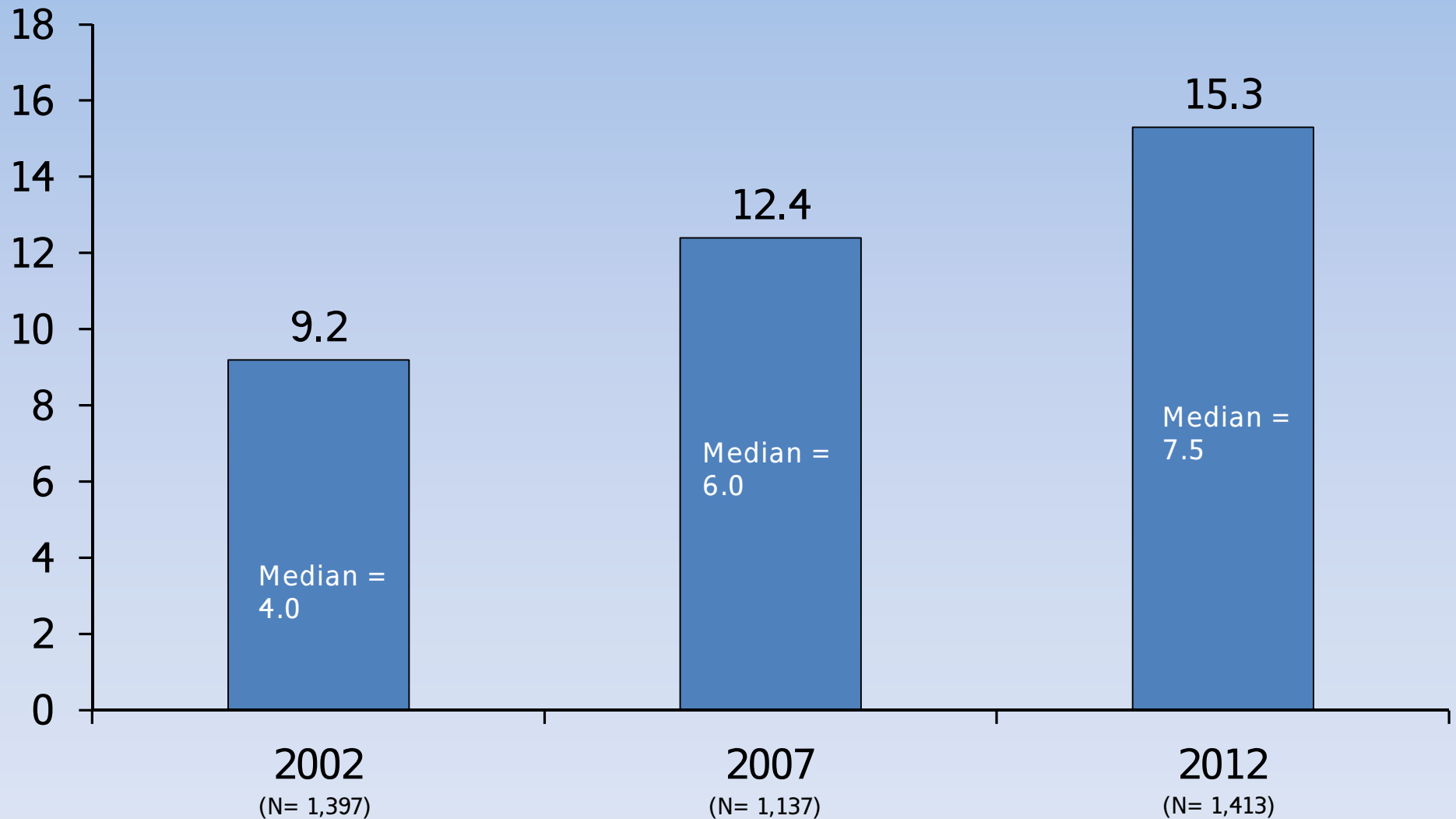
Current IgG Use: 1996, 2002, 2007 & 2012 IDF Patient Surveys and Community Surveys



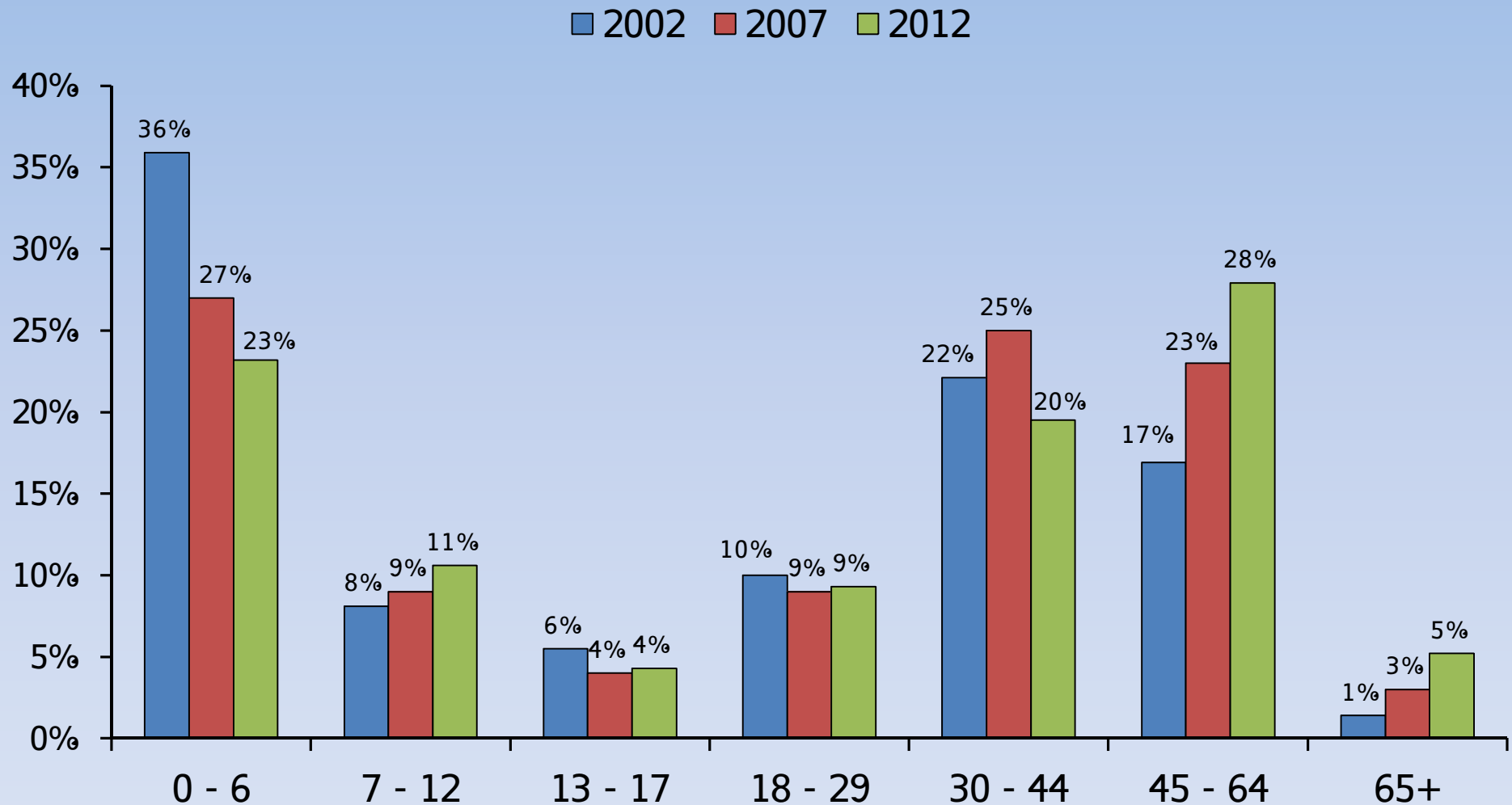
Q. Is the patient currently being treated with Ig therapy?

Base: All patients

Average Time to Diagnosis from Symptom Onset: All Diagnoses

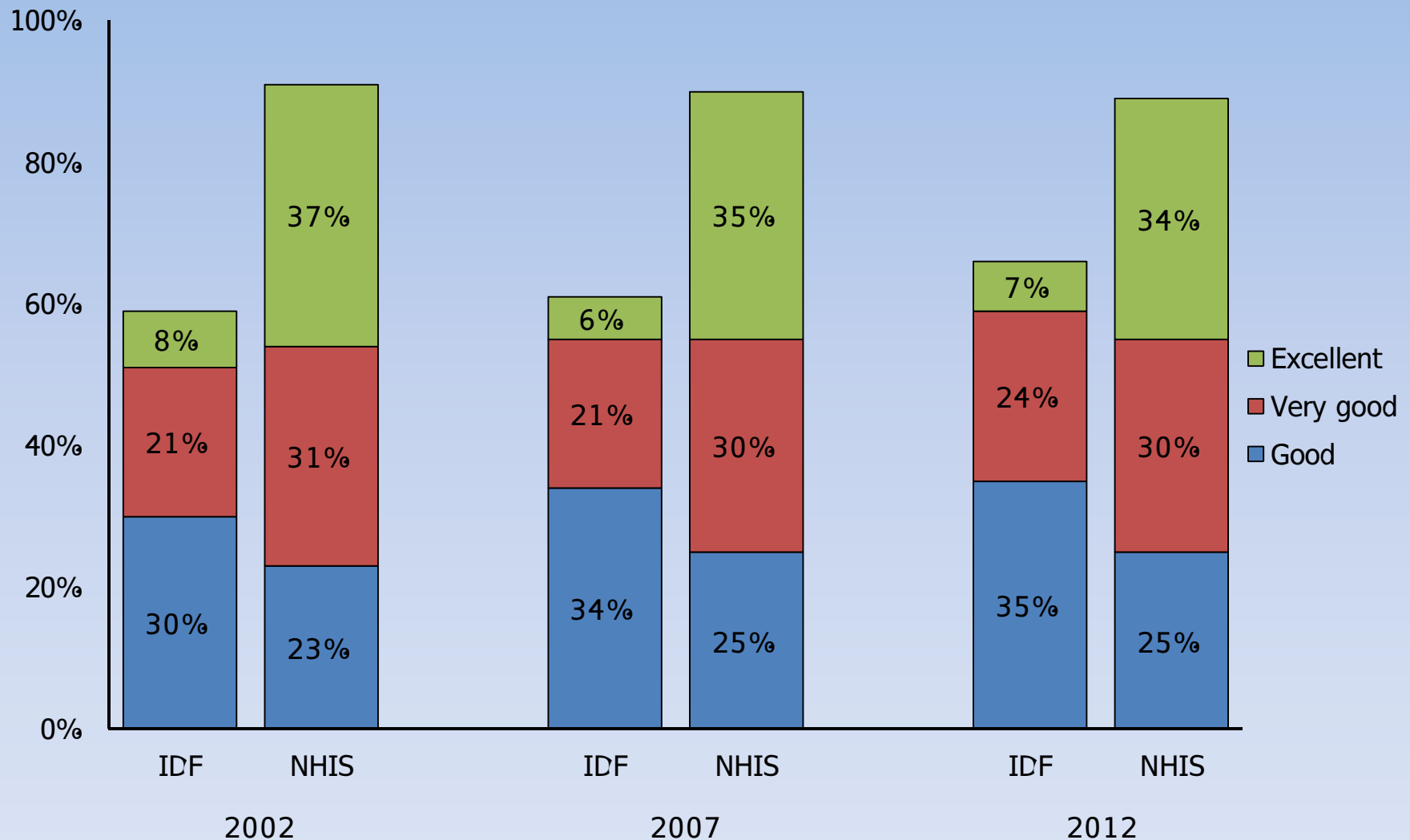


Age at Diagnosis



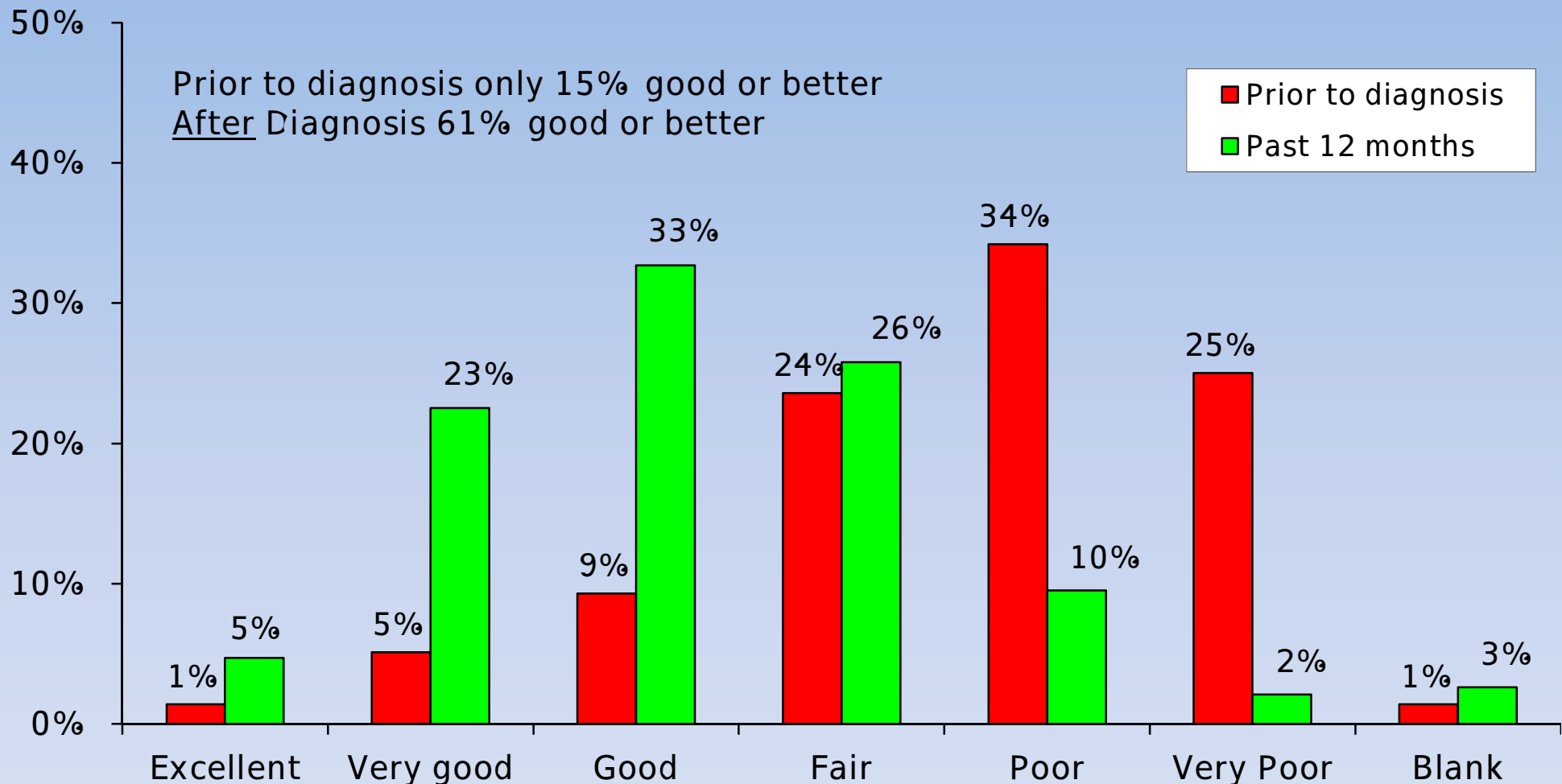
Sources: 2002, 2007 & 2012 IDF National Patient Surveys

Current Health Status: Good or Better



Sources: 2002, 2007 & 2012 IDF National Patient Surveys/
2002, 2007 & 2011 National Health Interview Survey (NHIS)

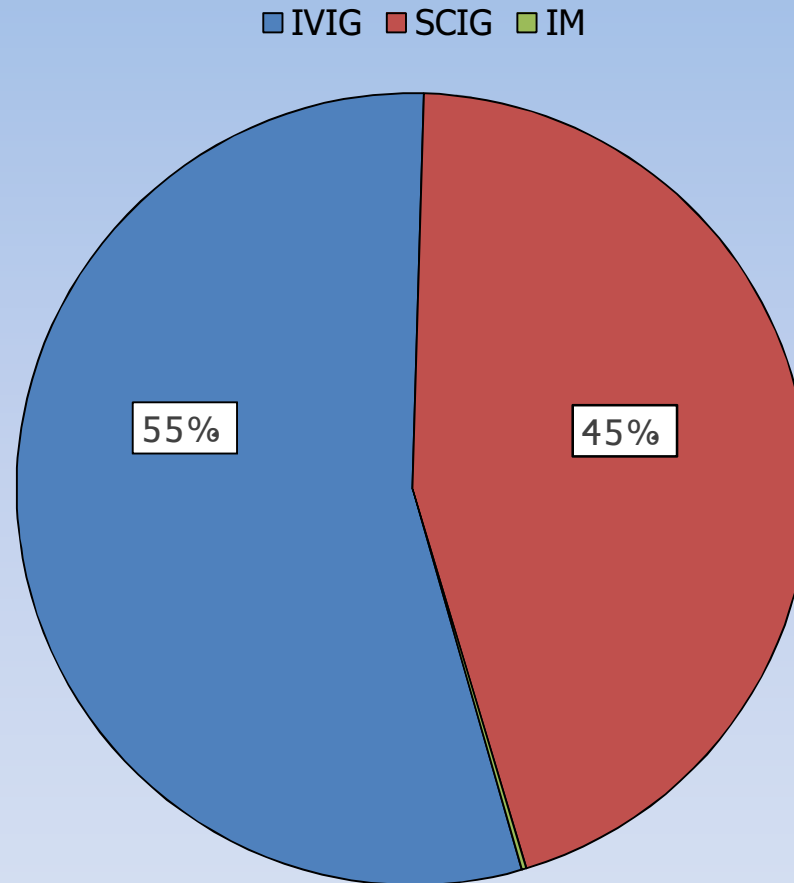
Health Status Improves after IG Therapy



Q9. Would you describe his/her health in the 12 months prior to diagnosis.....? BASE: Those who are currently using IVIG or SCIG Therapy N=1,428

Q61. Would you describe his/her health in the past 12 months as.....? BASE: Those who are currently using IVIG or SCIG Therapy N=1,428

Mode of Ig Therapy



Q21a. Is he/she currently being treated with SCIG, IVIG or IM for his/her immunodeficiency disease? Base: Those reporting current Ig use; N=1437

IDF 2013 Treatment Survey - SF Comparison for Total Sample

Report Date: 8/12/2014

Report Type: [SF Comparison for Total Sample](#)

Survey: [SF-12v2® Health Survey](#)



Report Criteria

Date Range: 1/15/2014 - 1/15/2014

Timepoint: All Timepoints

Demographic Profile

Sample Size: 1277
Male (*): 27% (339)
Female (*): 73% (912)
Mean Age: 51
Age Range: 3 - 99

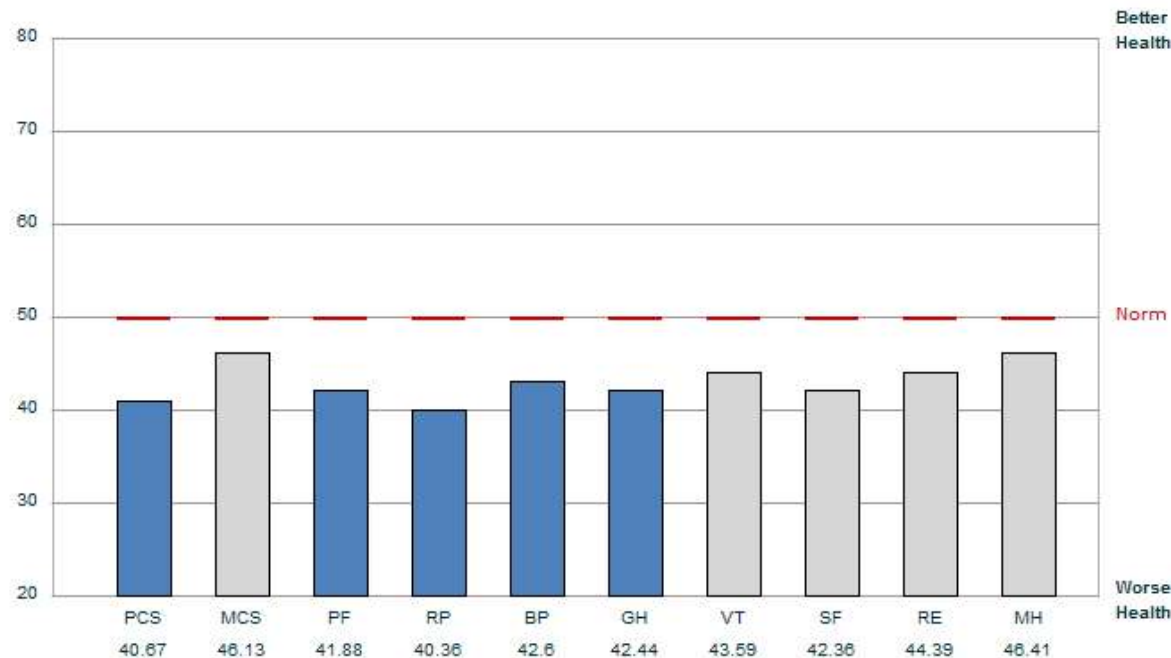
*Of those reporting gender (n=1251)

Comments

[Print this report](#) [Instructional Guide](#)

Scores for Total Sample

Physical Health Scores Mental Health Scores



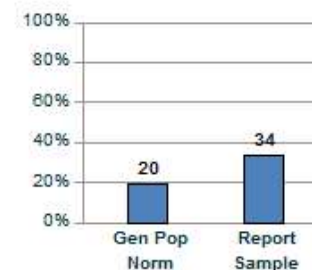
Abbreviation

PCS = Physical Component Summary
MCS = Mental Component Summary
PF = Physical Functioning
RP = Role Physical
GH = General Health

BP = Bodily Pain
VT = Vitality
SF = Social Functioning
RE = Role Emotional
MH = Mental Health

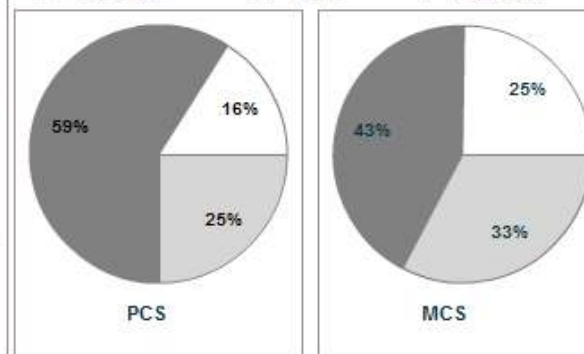
First Stage Positive Depression

Screening: % at Risk



% Sample whose Scores are Above, At or Below the General Population Norm

% Above % At % Below



Note: Totals may not equal 100%, due to statistical rounding.



United States Immunodeficiency Network

- The United States Immunodeficiency Network (USIDNET) oversees a Patient Consented Registry of Patients with PI.
 - Originated as CGD Registry in 1992, expanded to include all diagnoses in 1998
 - Funded by National Institute of Allergy and Infectious Diseases (NIAID)
 - Governed by a Steering Committee of top immunologists
 - Administered as a program of IDF
- Physician-validated clinical data on over 3,800 patients with PI
 - Laboratory results
 - Genetic and molecular information
 - Diagnostic criteria
- Longitudinal Data
 - Incumbent upon clinicians updating patient records over time
 - Updates do not always happen

Case Study: Value of Blended Data

Autoimmunity and Inflammation in XLA*

- Two sources: USIDNET and Patient Reported IDF Survey
- USIDNET Steering Committee- *Hypothesis-Incidence of AI conditions in registry among patients with XLA is under reported*
 - Data from IDF survey of patients with XLA compared to data on XLA patients in USIDNET registry.
 - Patients reported higher incidence of diagnoses of AI and higher incidence of symptoms consistent with an AI diagnosis than what was found in the registry.
 - Use of both a patient survey and USIDNET Registry data was important in gaining a better understanding of the association of inflammatory disease in XLA.

USIDNET vs. XLA Survey Reported Conditions

Condition	USIDNET Registry (N=149)	XLA Patient Survey (N=128)	p value
Aches, malaise or fatigue	22 (14.8)	44 (34.4)	P < .001
Pain, swelling, arthralgia	18 (12.1)	28 (25.0)	P = .035
Abdominal pain	10 (6.7)	22 (17.2)	P = .008
Diarrhea	13 (8.7)	27 (21.1)	P = .005
Chills	1 (.7)	17 (13.3)	P < .001
Shortness of breath	1 (.7)	21 (16.4)	P < .001
Constipation	3 (2.0)	14 (10.9)	P = .002

eHealth evolution for PI

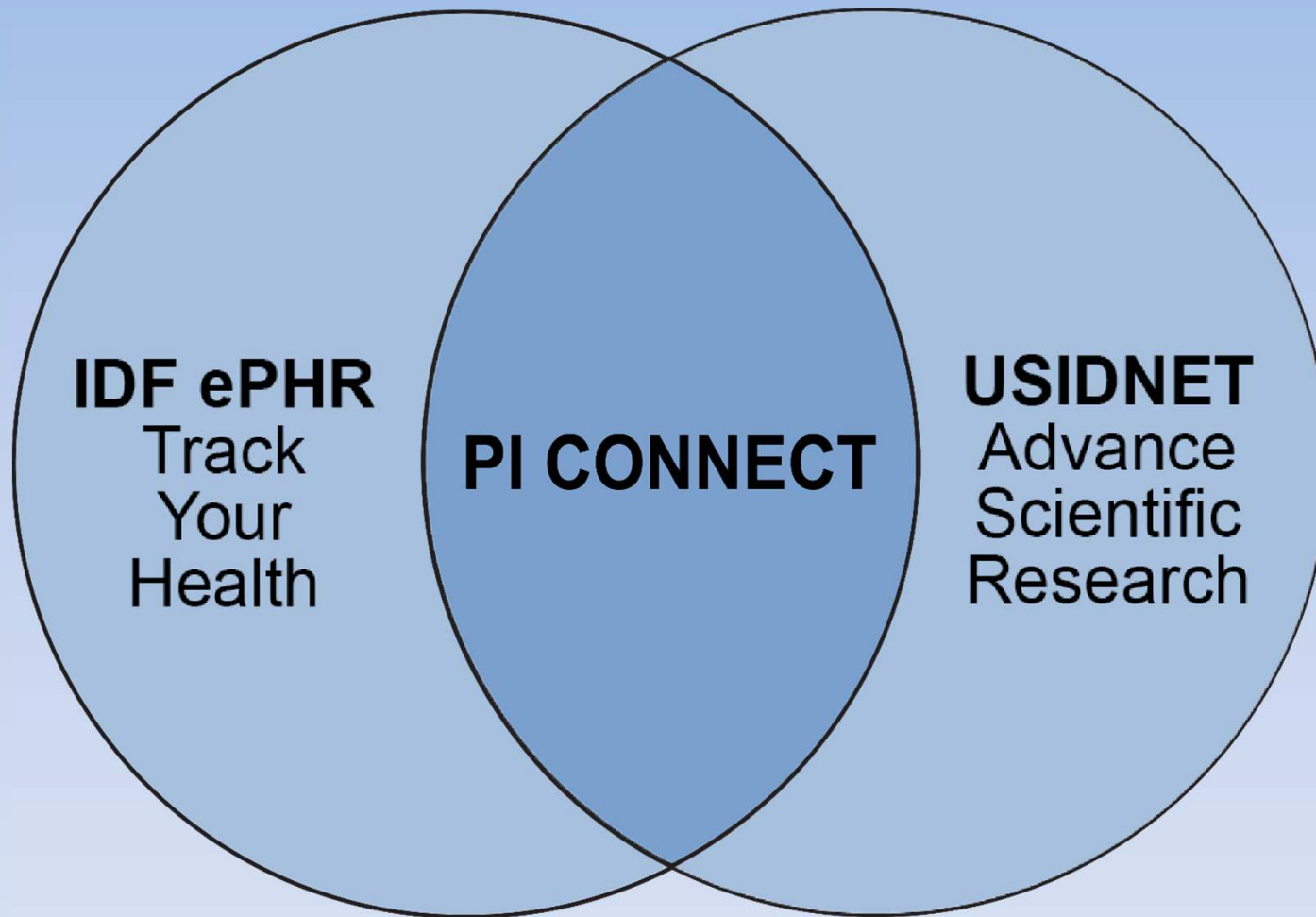
- 2011- IDF launched an online electronic personal health record (ePHR)
 - Developed for individuals and families living with primary immunodeficiency diseases
 - Patients track their symptoms, record medications and log health information
 - No cost for patients and family members
- Fall 2014 - IDF transformed ePHR with new features, including cutting edge tracking and reporting tools:
 - Sync with Microsoft HealthVault
 - Set health and fitness goals and track progress
 - Quality of Life survey instruments
 - Connection to PI CONNECT

www.idfePHR.org

PI CONNECT: Combining Registry Clinical Data and Patient Reported Data

IDF was granted an award from the Patient Centered Outcomes Research Institute (PCORI) to create PI CONNECT, a patient-powered research network that is part of the national patient-centered clinical research network.

PI CONNECT



PI CONNECT

This opportunity allows IDF to create a premier research network for the PI Community by doing the following:

- The IDF ePHR acts as a portal for patients to electronically consent into the USIDNET Patient Registry
- Users update their ePHR with patient generated data. This de-identified data is combined with the physician validated clinical data in the Registry.
- Researchers now have both sets of data for a clearer picture of the patient experience
- PI Connect Research Online Forum acts as a venue for patients to share research ideas with researchers, and to comment on research queries to the USIDNET Registry

Thank You!