

# Emerging Commercialization Strategies for Addressing the Needs of Ultra-Orphan Patients

*September 27 – 29, 2010 | St. Louis, MO*

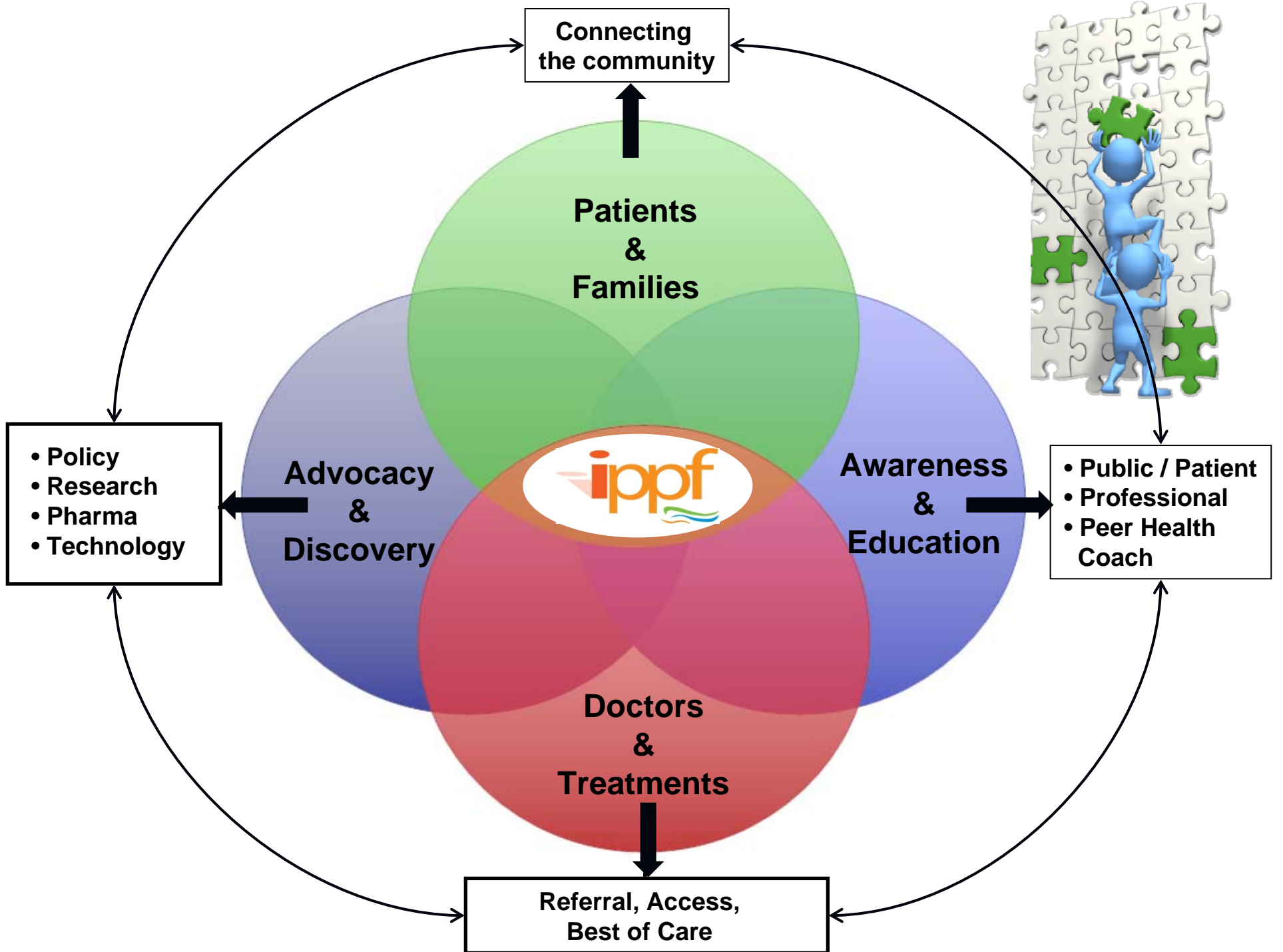


## **Patient Centered Health Management / REMS 2.0**

Pemphigus Vulgaris and related bullous disorders:  
Outcomes of a model for improving Ultra-Orphan illness  
through an individualized Health Management Program.



David Sirois DMD, PhD  
President, Board of Directors  
International Pemphigus and Pemphigoid Foundation



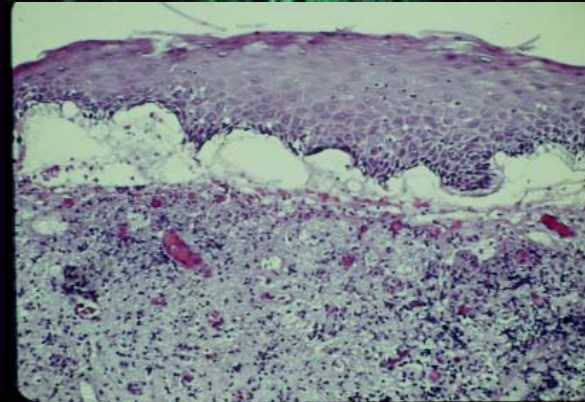
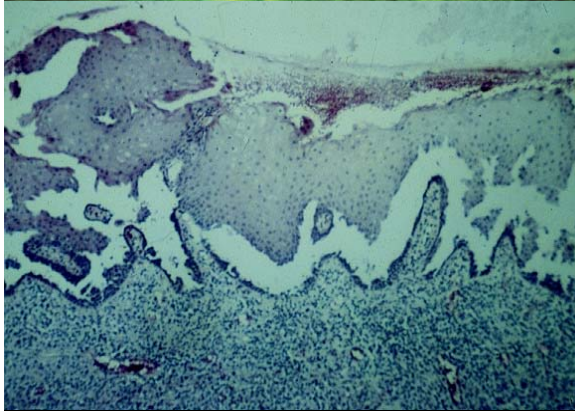
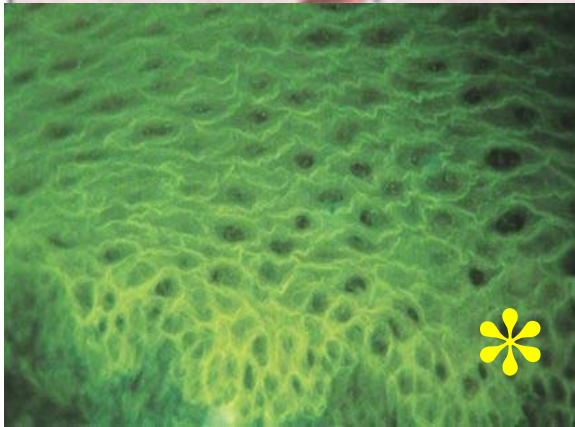
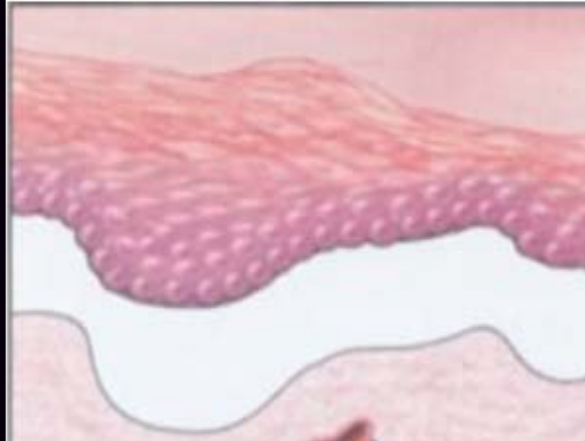
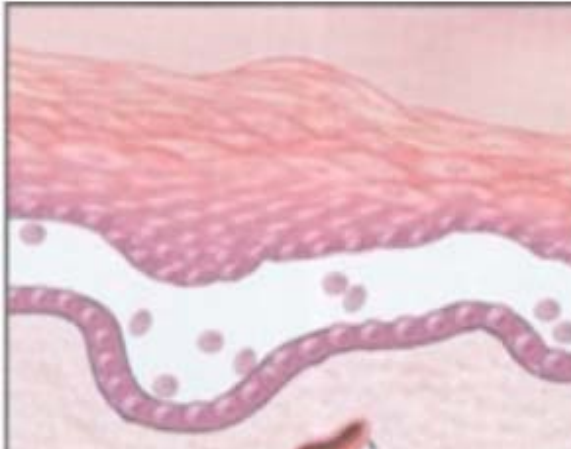
# HOW WE PROVIDE SUPPORT

- People who care: our volunteers and staff
  - Website, E-Bulletins, Newsletters
  - Electronic Patient Forums
  - Email discussion groups and chat rooms
  - Library of educational materials
  - Medical Advisory Board
  - Annual Patient Meetings
  - Supporting Scientific meetings
  - Coalitions
  - Partnerships
- ❖ Award winning
  - ❖ >110,000 page views/yr
  - ❖ >7,000 unique visitors
  - ❖ Facebook
  - ❖ Youtube
  - ❖ Twitter

# Pemphigus and Pemphigoid

- Ultra orphan illness with estimated 30,000 affected in U.S.
- Autoimmune disorders that destroy proteins that hold together the skin and mucous membranes
- The result is multiple, chronic ulcerations and erosions of the skin an/or mucosa
- Morbidity and mortality





**pemphigus**

**pemphigoid**



# Treatments

- Glucocorticoids
- Mycophenolate
- Mofentil
- Azathioprine
- Methotrexate
- Cyclophosphamide
- Cyclosporin
- IVIg
- Rituxmab
- Etanercept
- Infliximab
- Tetracyclines
- Dapsone
- Cholchicine
- Plasmaphoresis

# Living with pemphigus and pemphigoid

- Pain
- Lesions / wounds
- Infection
- Fear
- Anxiety, Depression
- Disability
- Appearance
- Medications
  - Costs & Access
  - Side Effects
  - Complications
  - Chronicity
  - Diabetes, hypertension, osteoporosis, adrenal suppression, immune suppression, marrow suppression, infection, GI, hepatic and renal toxicity,

# Advocacy – Industry Partnership Opportunities:

## *The special case of orphan / ultra-orphan illnesses*

- Though orphan illnesses affect <200,000 persons, and ultra-orphan <50,000... in aggregate 30M Americans suffer with at least 1 of 7,000 rare disorders: collectively affect 1 in 10 people in the U.S .... and many are chronic illnesses
- Patient Advocacy Groups have access, “touch”, and mission
- Pharma, academics, payors and policy makers have desire / need to understand population needs, status and opportunities
- Advocacy, discovery, delivery, surveillance, outcomes, compliance (REMS)

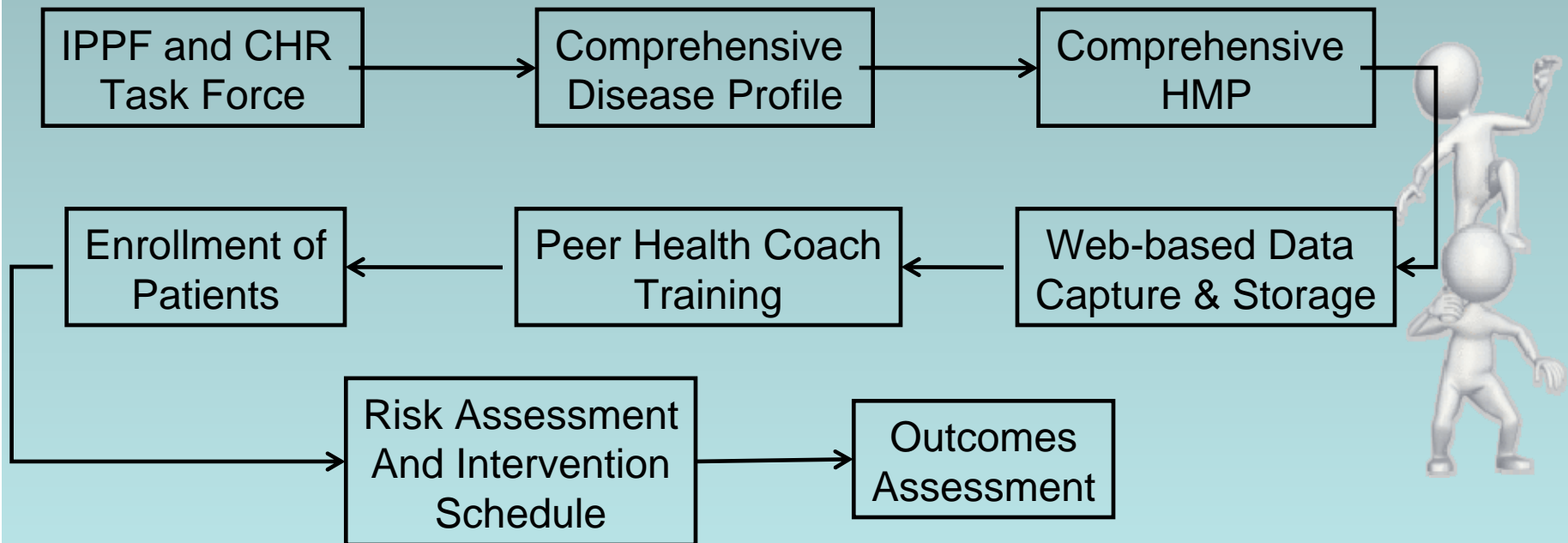




International Pemphigus and Pemphigoid Foundation



# Health Management Program





International Pemphigus and  
Pemphigoid Foundation



## Health Management Program Goals



1. Achieve and maintain remission, and manage relapses
2. Reduce patient's risks from prolonged or high dose steroid use
3. Decrease the number of adverse drug events (ADE's)
4. Improve patient's oral hygiene, and improve early identification of nasal, laryngeal and pharyngeal disease
5. Reduce occurrence or magnitude of secondary infection
6. Detect and improve psychosocial disorder
7. Improve quality of life and promote health lifestyle

- **Participant benefits:**

- Education about illness, medications, and therapies
- Learning better communication with health care team
- Achieving compliance with the treatment plan
- Developing self-care skills and coping and lifestyle support
- Advocacy to overcome barriers to treatment

- **Physician benefits:**

- Enhanced physician-patient communications
- Happier, healthier, and empowered patients

- **Entire illness population benefits:**

- Improved compliance, quality of life, fewer adverse events, reduced side effects from steroids as a result of the program.
- Increased awareness and interest in our cause, with a goal of attracting more funding and more research to find a cure.

**HMP:** personalized assessment by PHC using structured interview, web-based electronic data capture, individualized risk assessment and management plan; measure outcomes.

Medication

<b>Segmentation</b>	<b>Disease Activity</b>	
	activity $\geq 5$	activity $< 5$
<b>Prednisone <math>&lt; 10\text{mg/d}</math></b>	Q 3m x 4, then Q 6m	Q yr
<b>Prednisone <math>\geq 10\text{mg/d}</math></b>	Prn, min Q m	Q 3m

ARE YOU ENROLLED?  
YOU SHOULD BE!

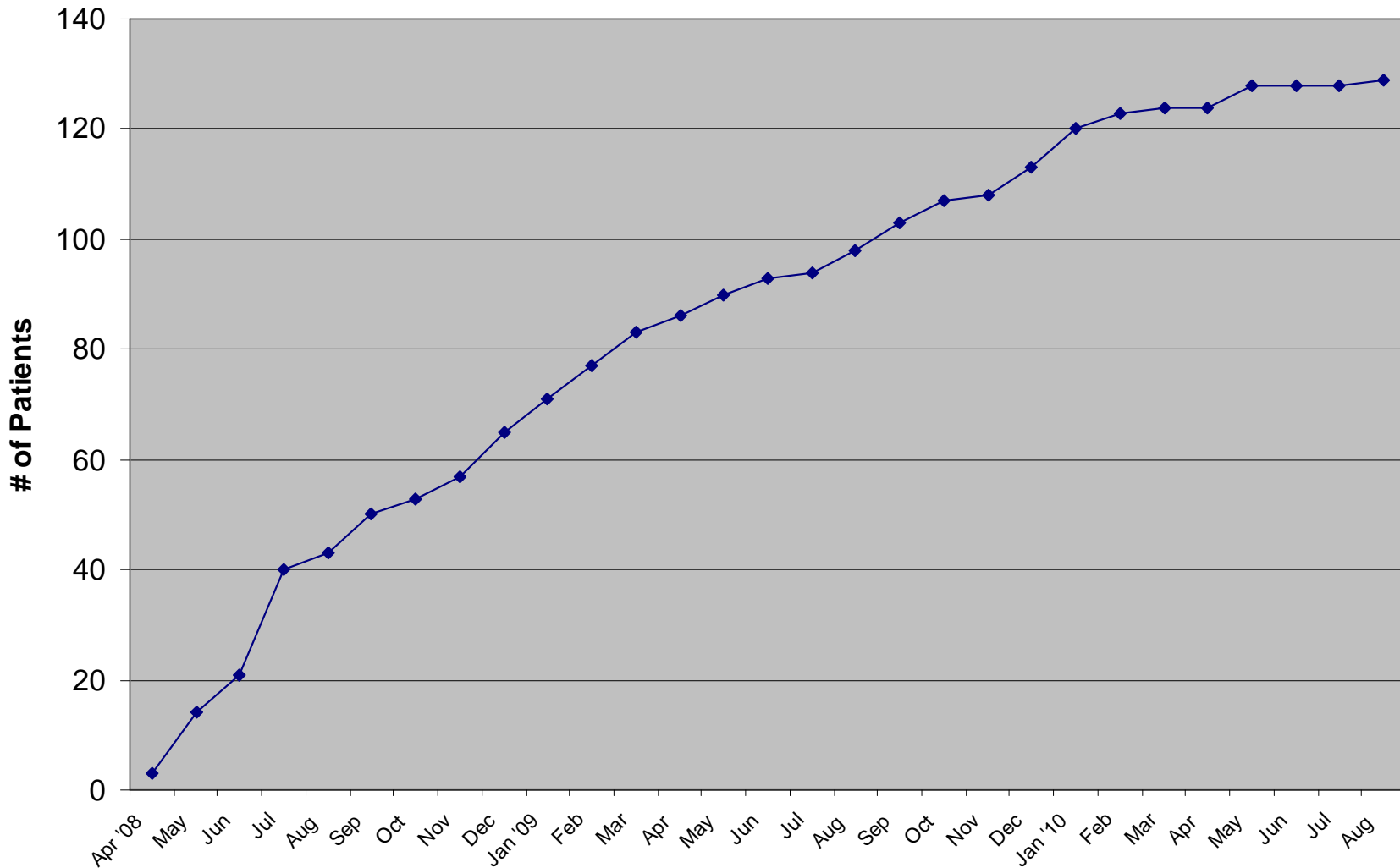
**HMP**  
Health Management Program

[CLICK HERE  
FOR MORE  
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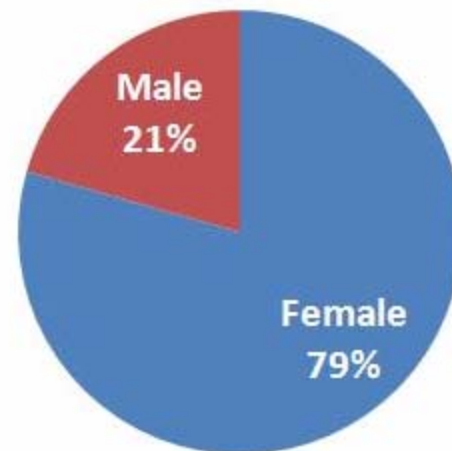
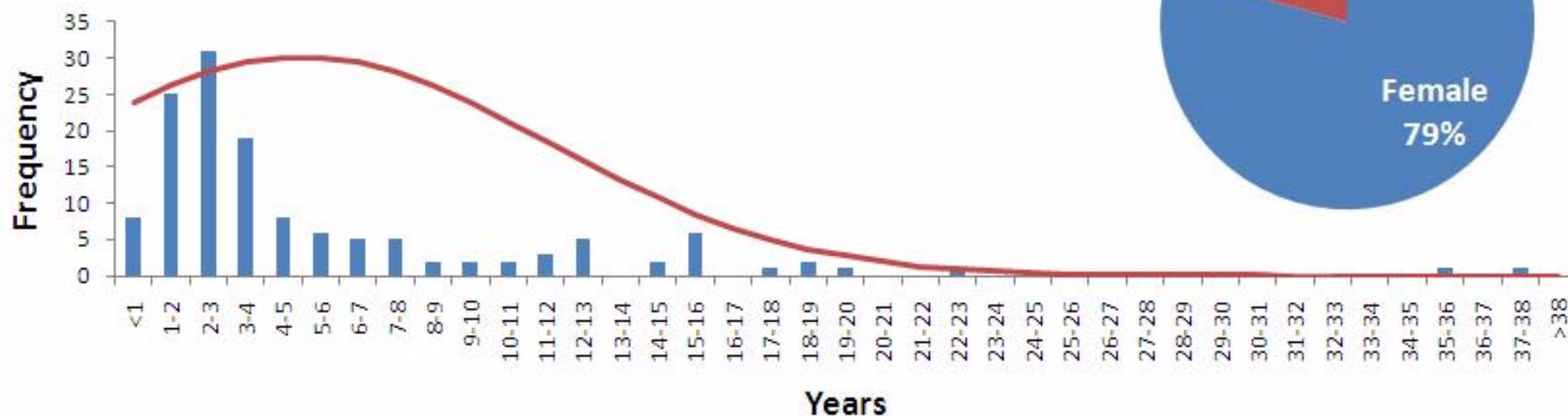
# HMP Outcomes

IPPF HM Program Enrollment (Active)

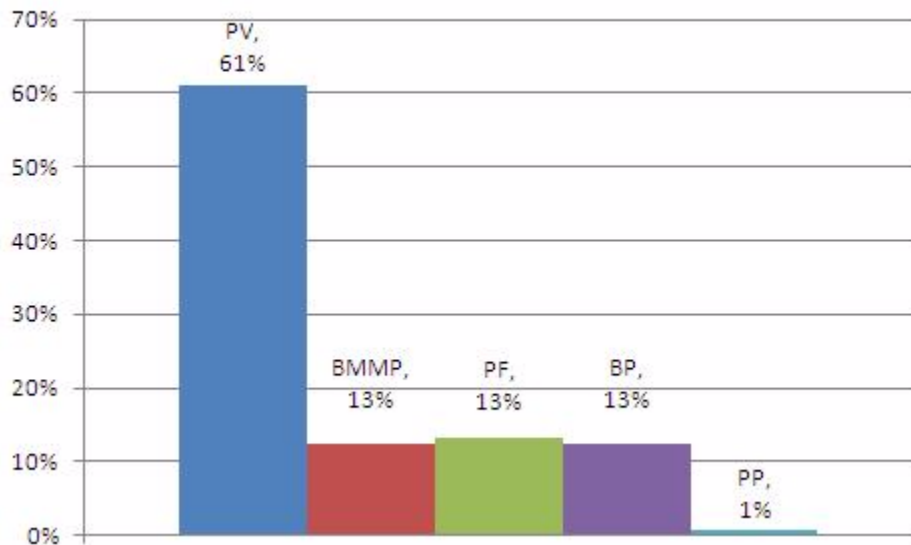


### Duration of illness

mean 5.5±6.6 yrs

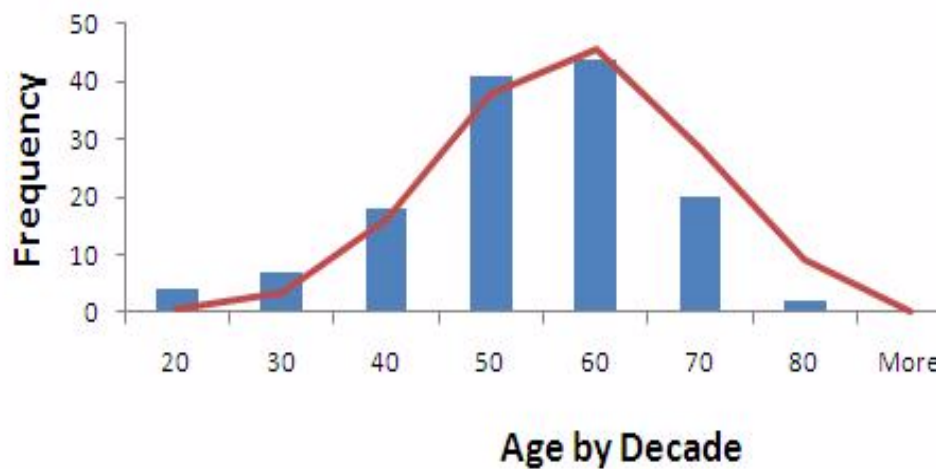


### Distribution of Bullous Disease

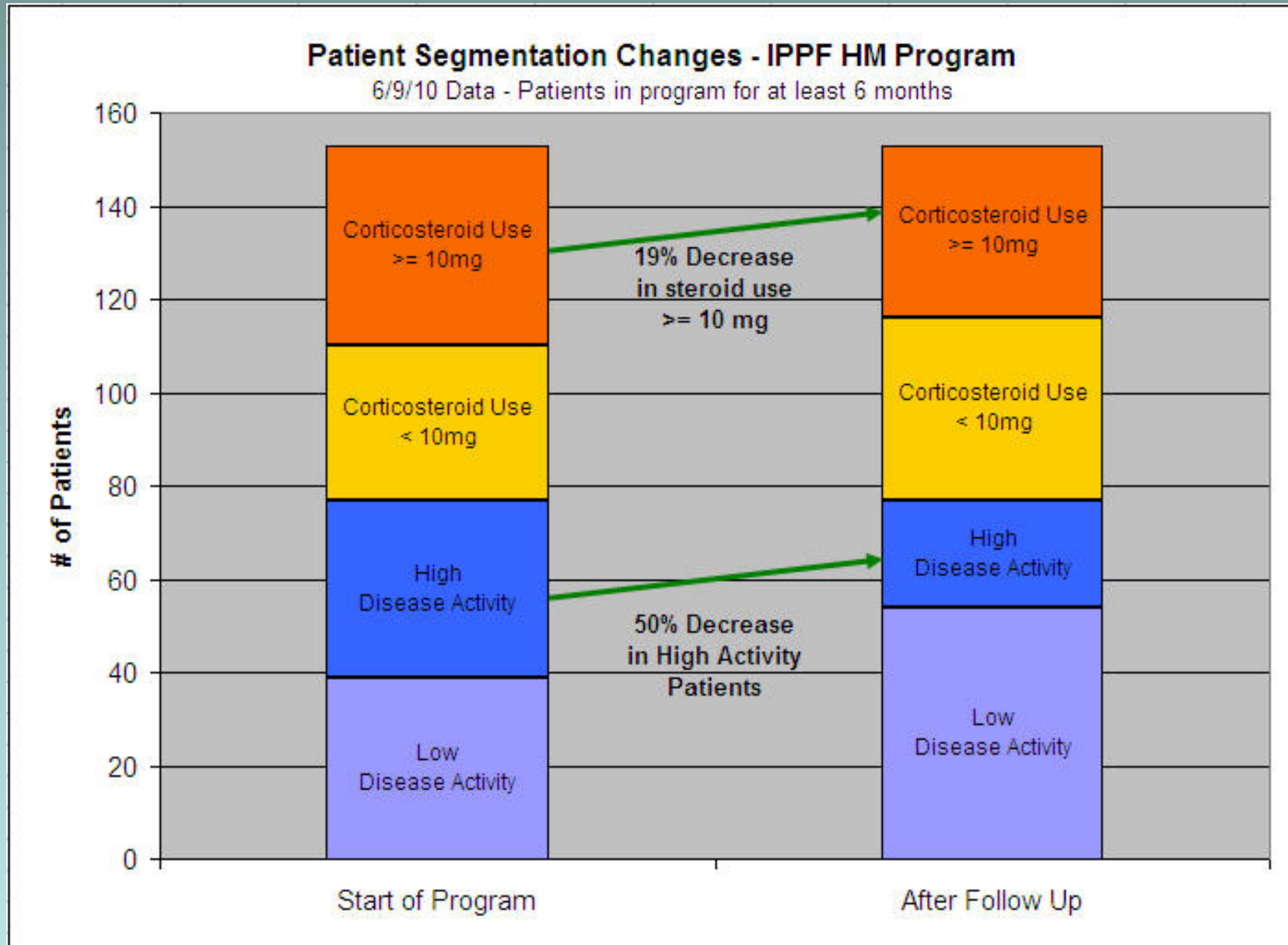


### Age at Diagnosis

Mean 57.9±12.3 years

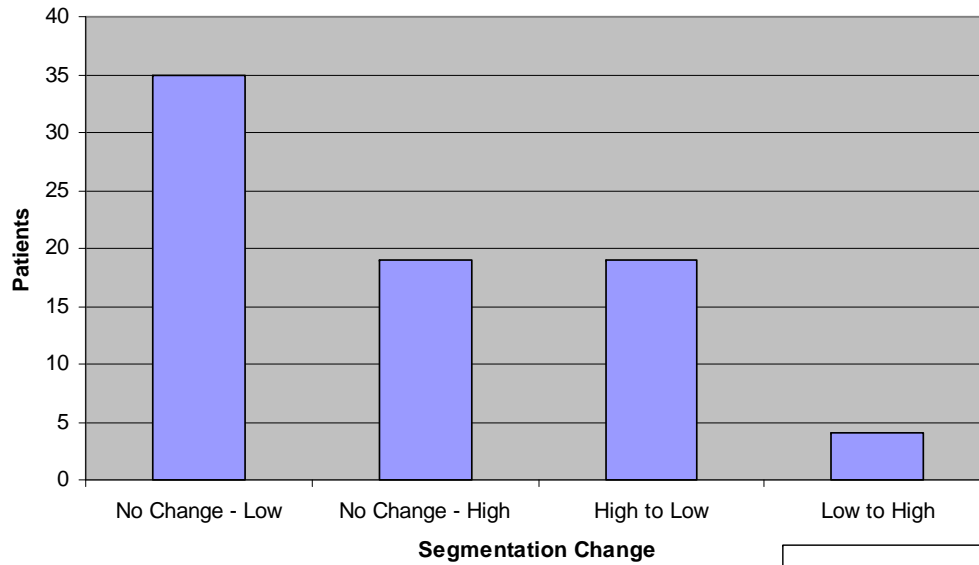


# Did Patients Change Segmentation for the Better?



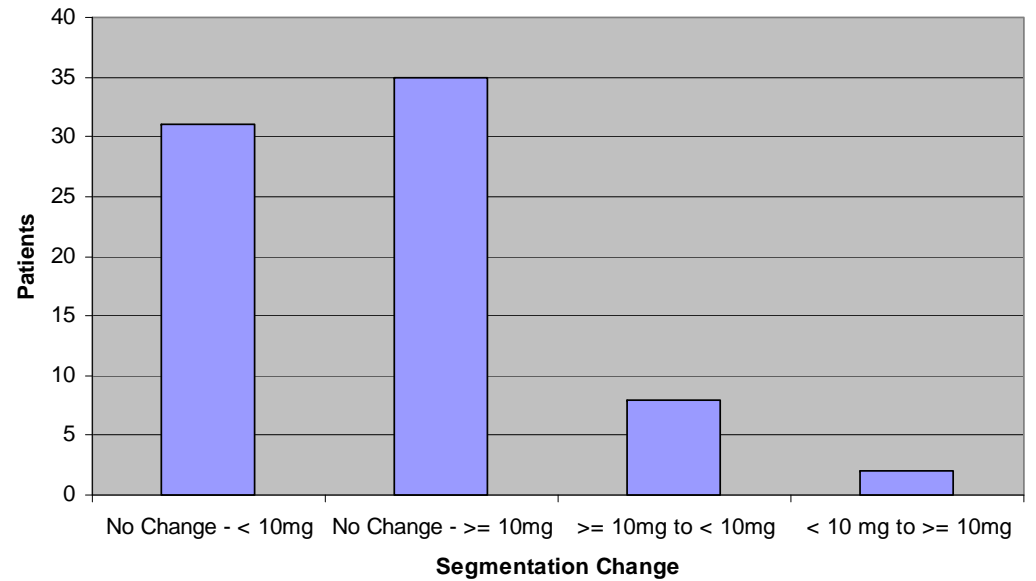
### Patient Segmentation - Disease Activity

50% of patients that started program with High Activity reduced to Low Activity



### Patient Segmentation - Corticosteroid Use

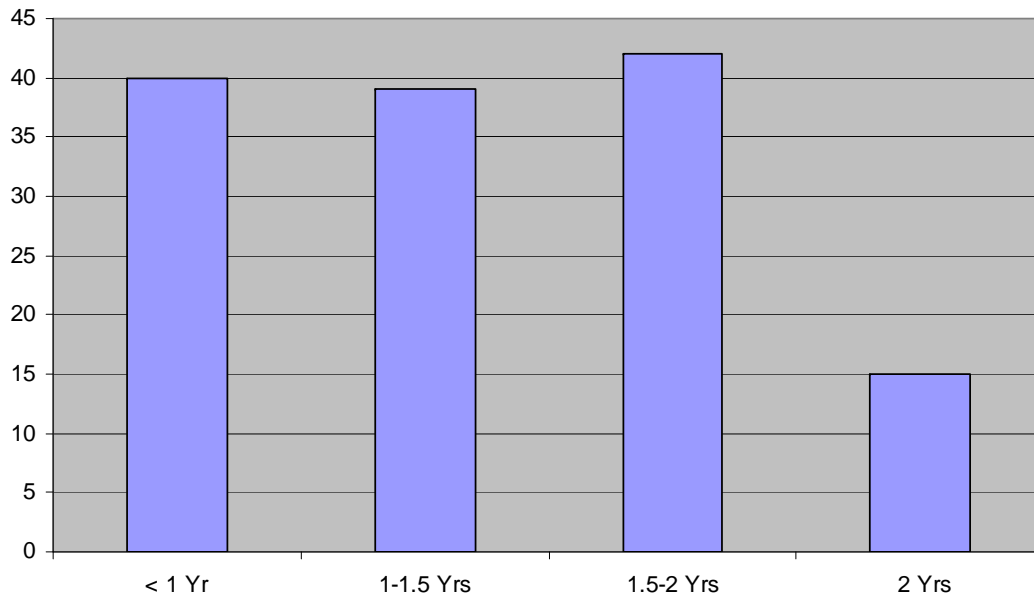
19% of patients that started program with  $\geq 10$  mg reduced to  $< 10$ mg



Another way to look at Segmentation Data

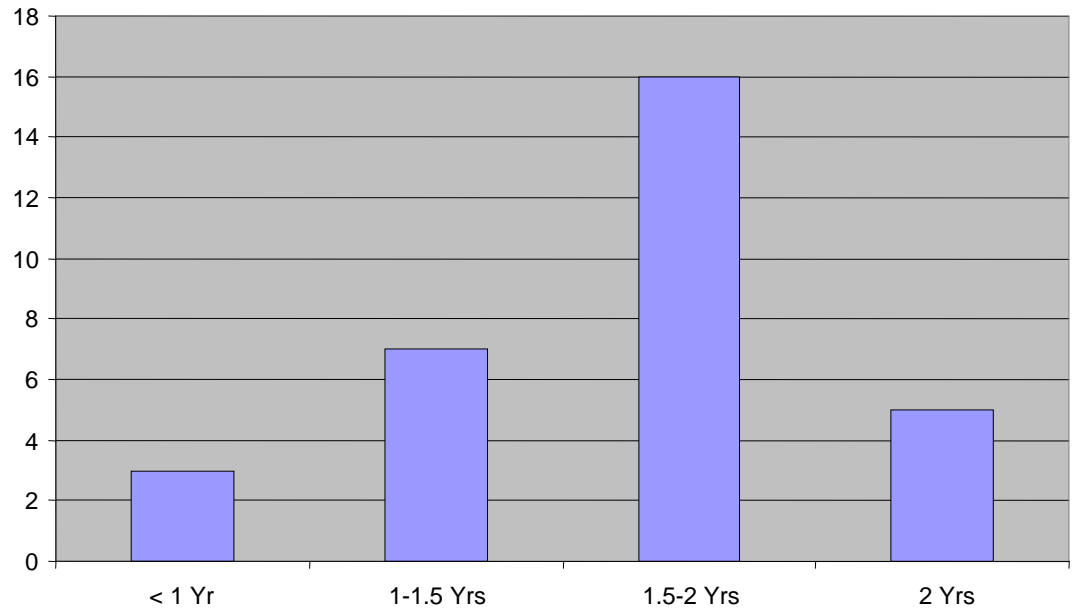
# Assessing impact of HMP

Time in HM Program - All Patients



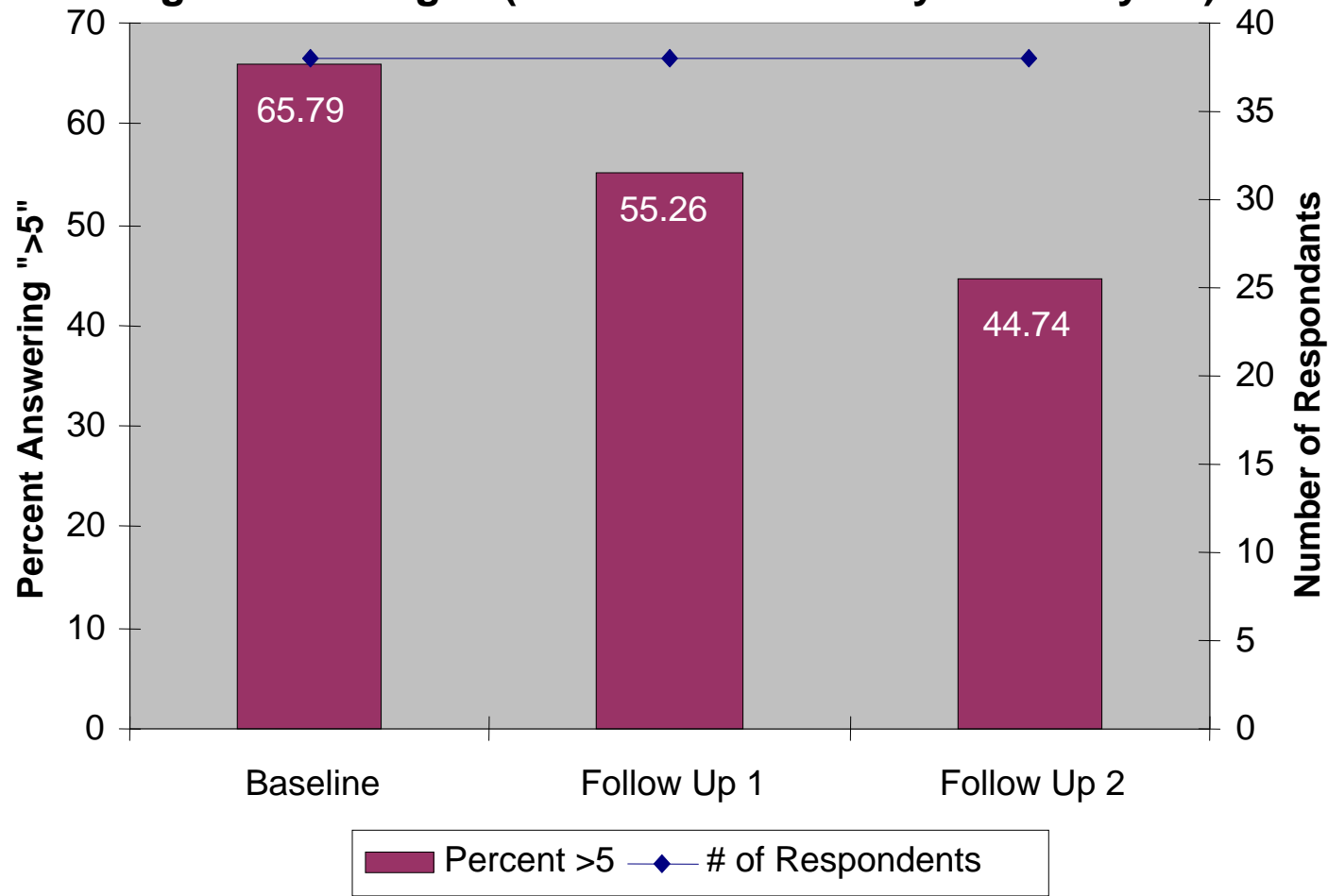
**Selecting HMP  
patients with 3+  
assessments**

Time in HM Program - 38 Patients



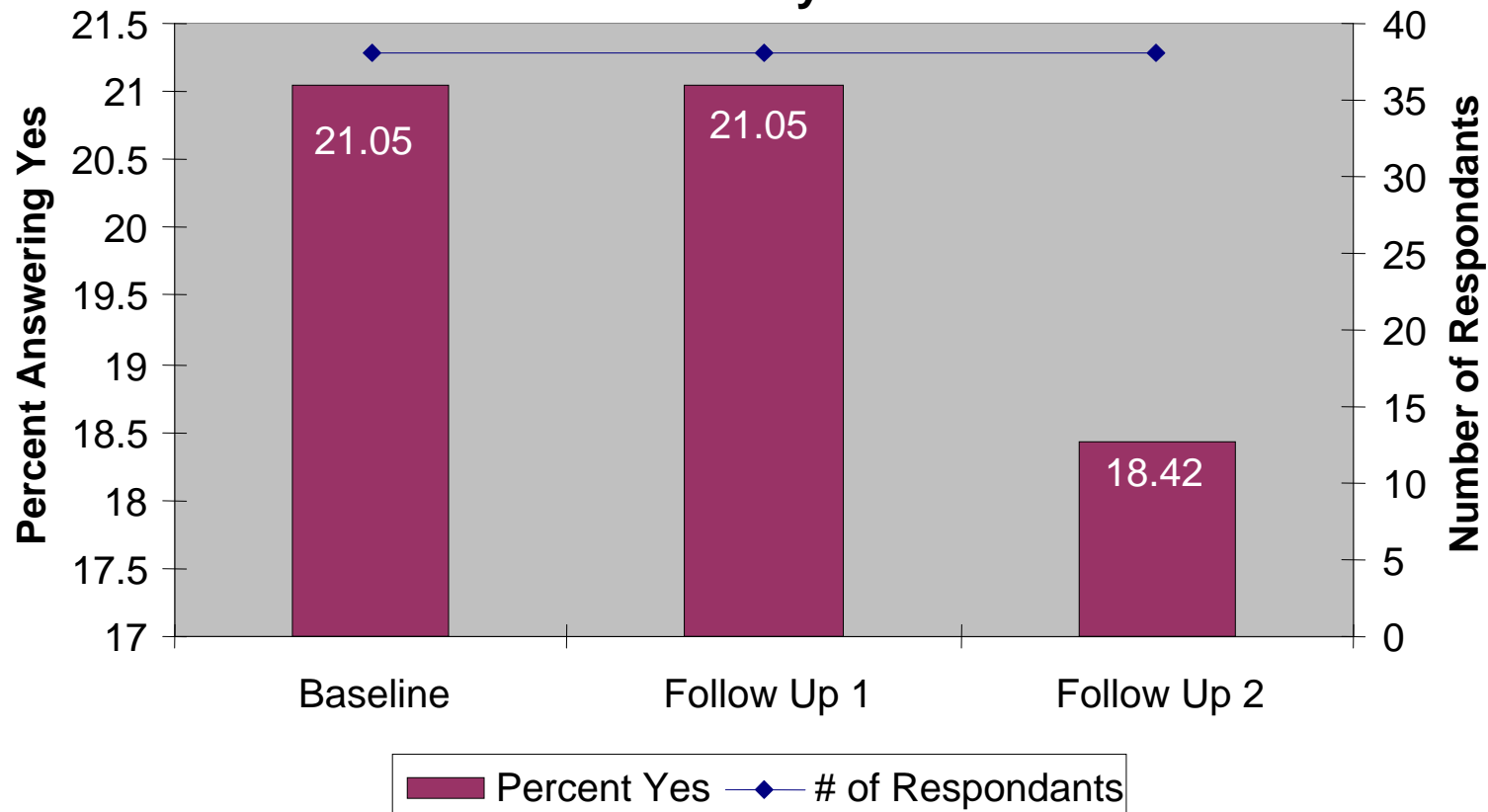
# 32% reduction in significant fatigue

Q61 - How many days in the last month have you had significant fatigue (that interferes with your lifestyle?)



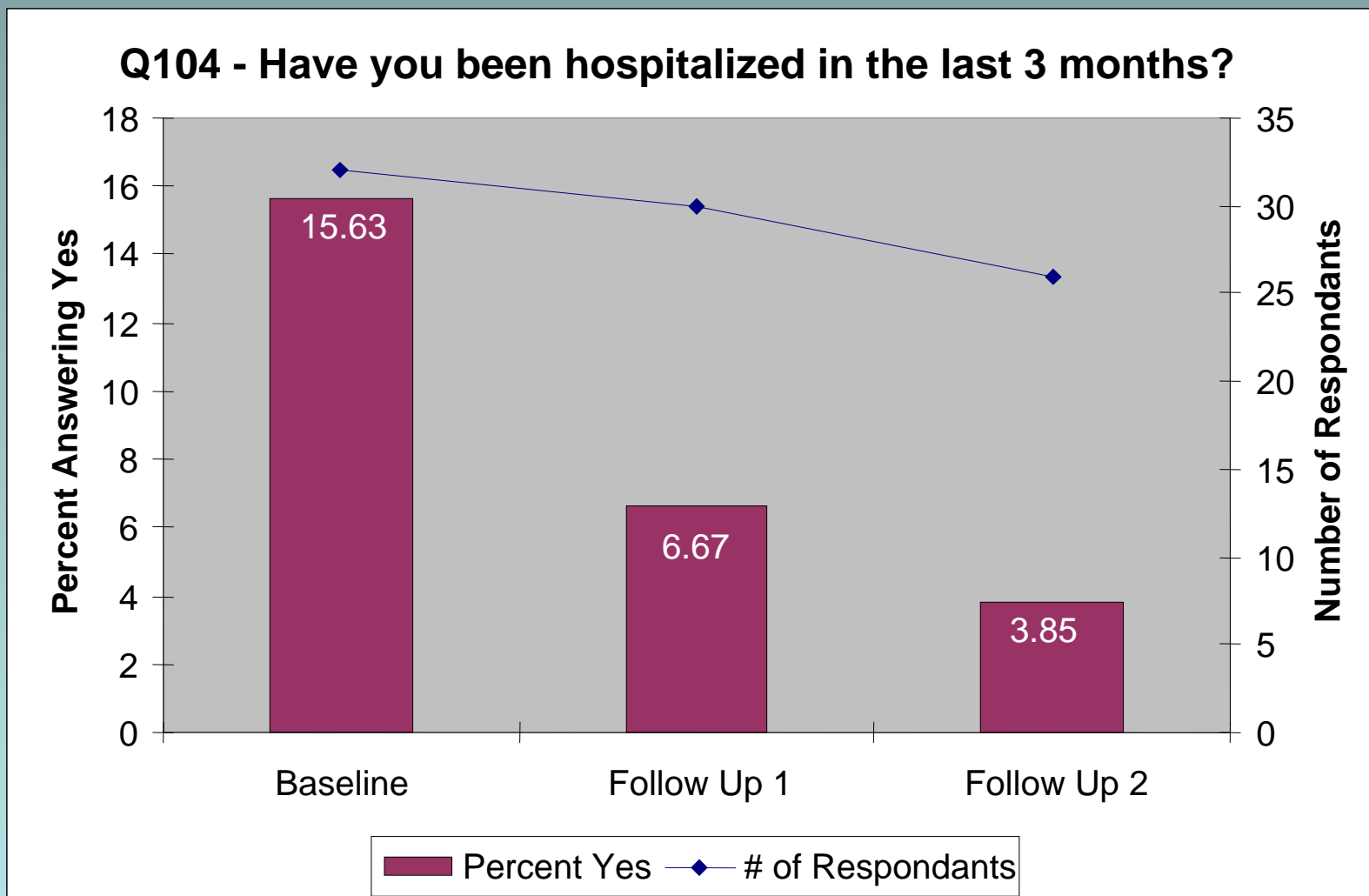
# 12.5% reduction in missed work

**Q143 - Have you missed any days from work due to your condition since the last time you received this survey?**

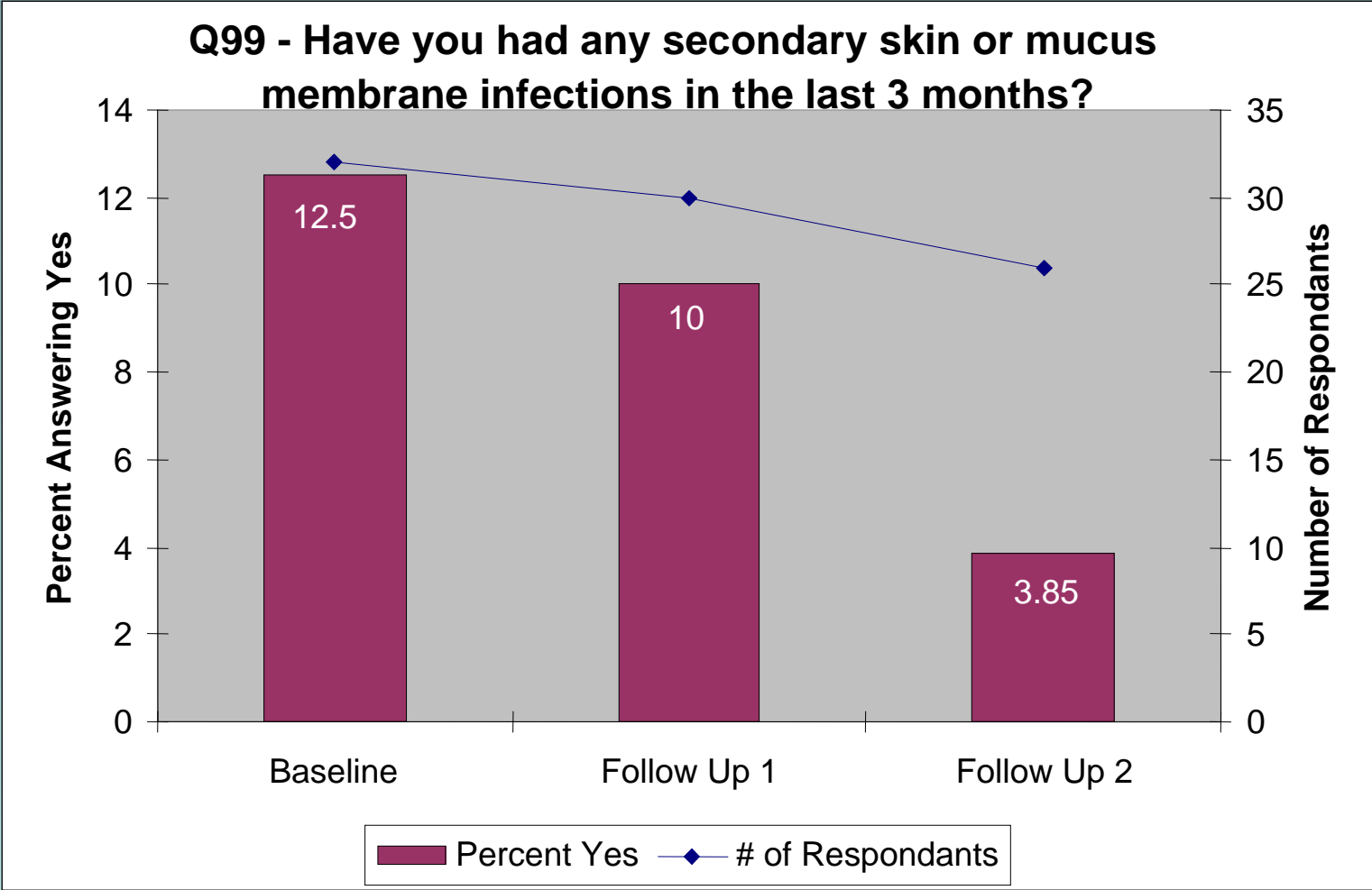


# 75% reduction in hospitalization

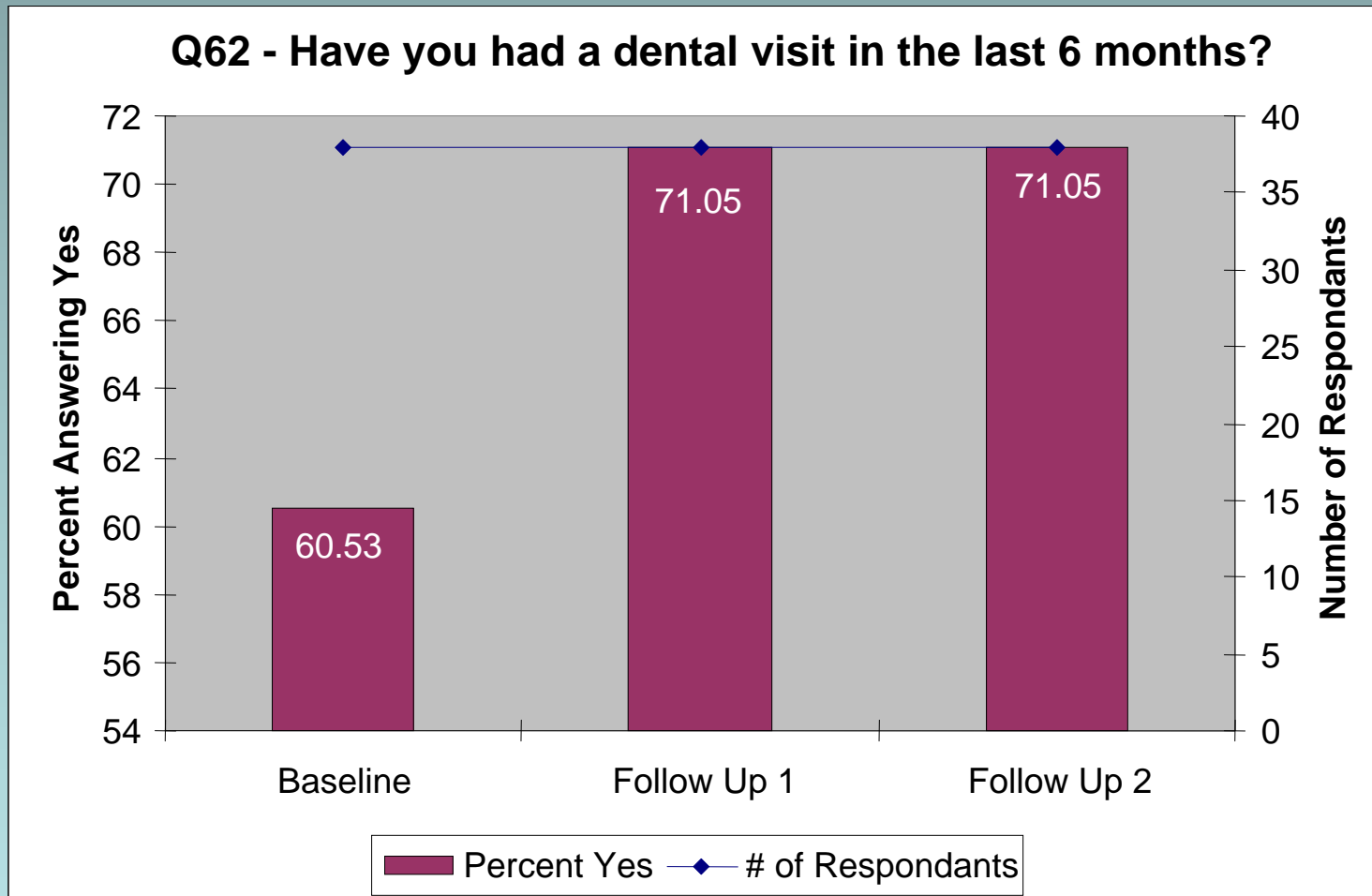
for those patients taking  $\geq 10\text{mg}$  prednisone/day



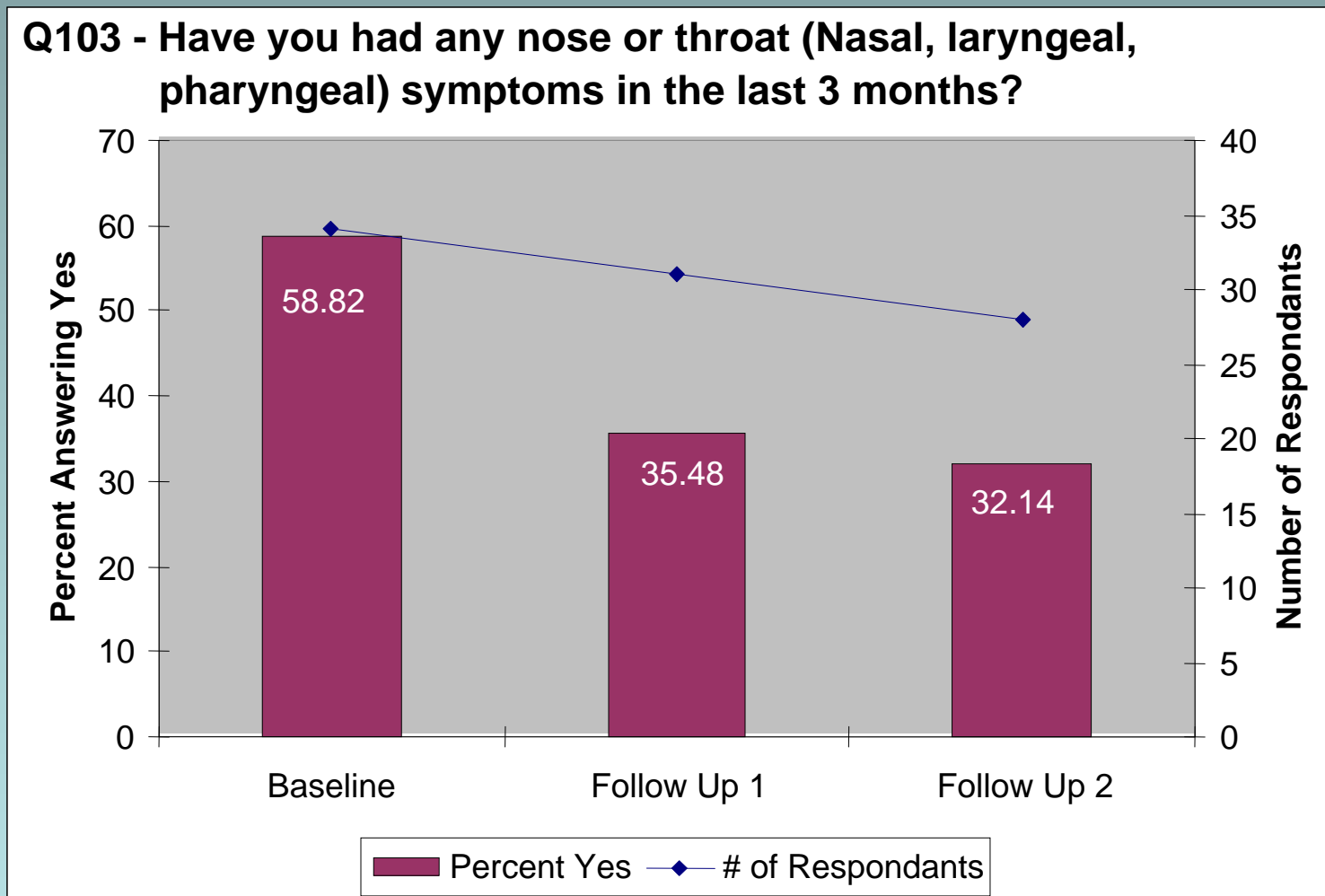
# 69% reduction in secondary infection for those patients taking $\geq 10$ mg prednisone/day



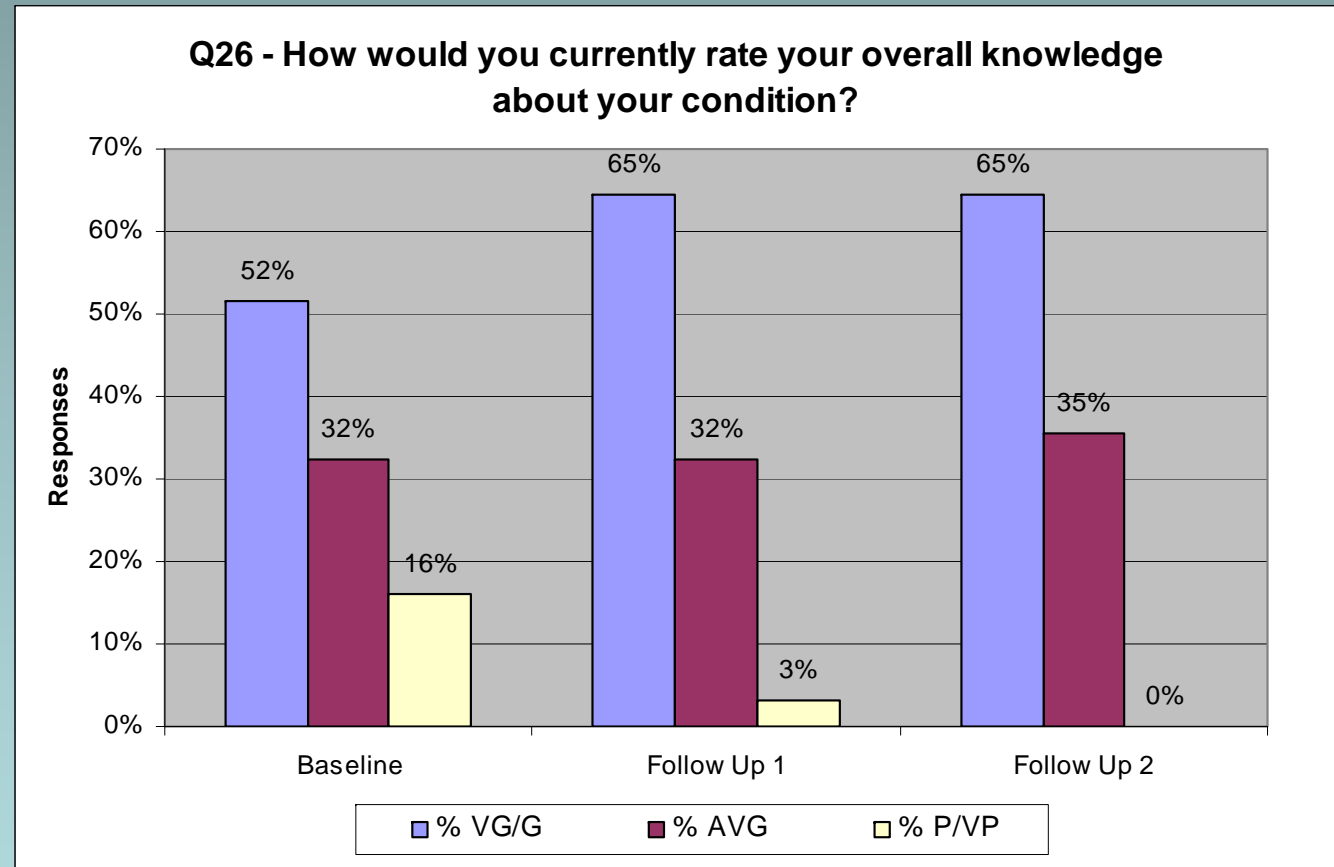
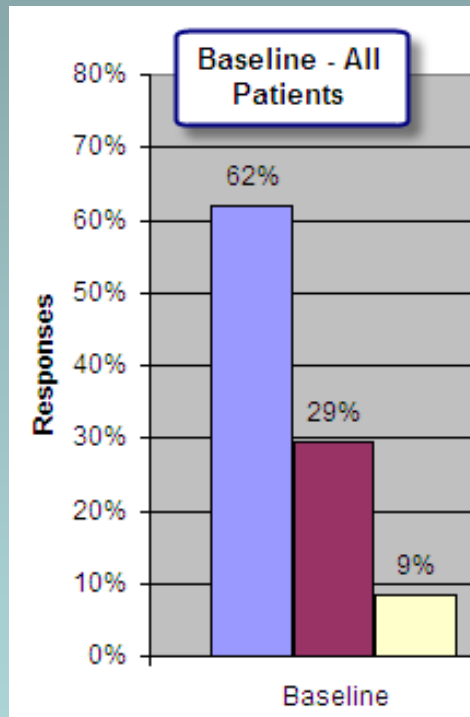
# 19% increase in oral health promotion compliance



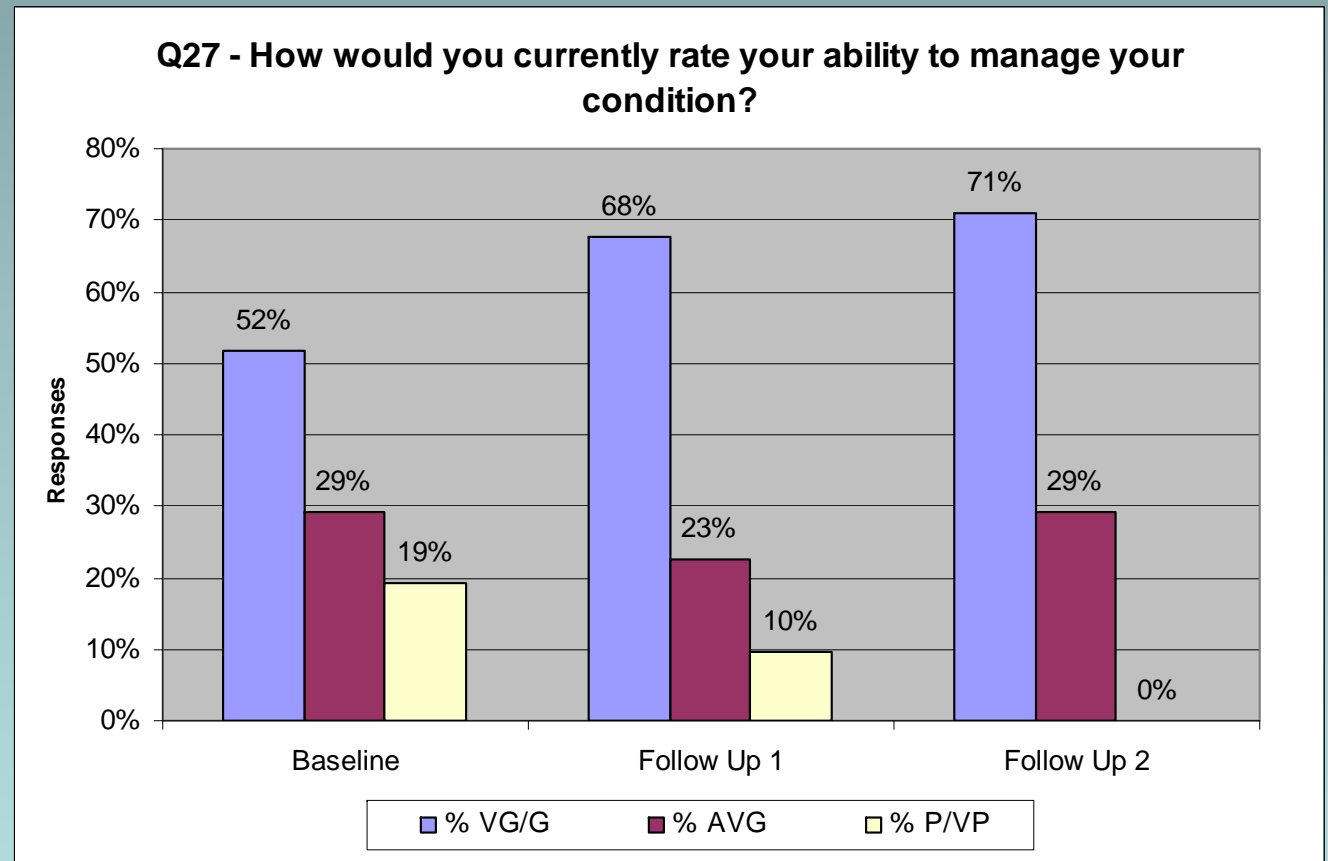
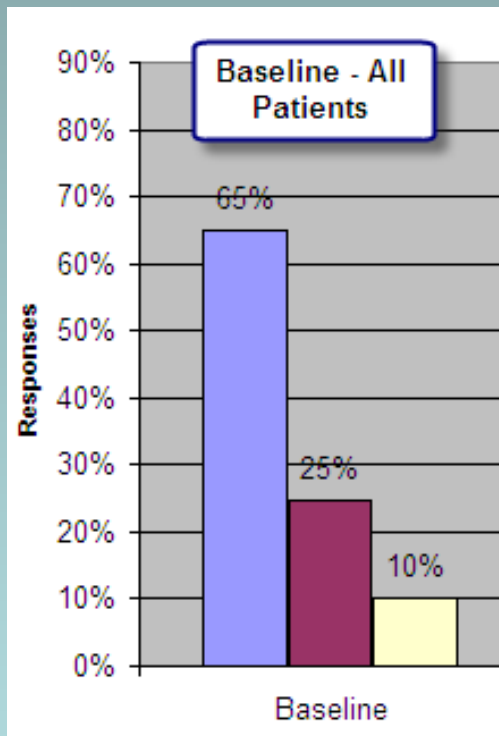
# 45% reduction in oropharyngeal symptoms for those patients taking $\geq 10$ mg prednisone/day



# No patients continue to rate their knowledge poor or very poor after 2 program follow ups!

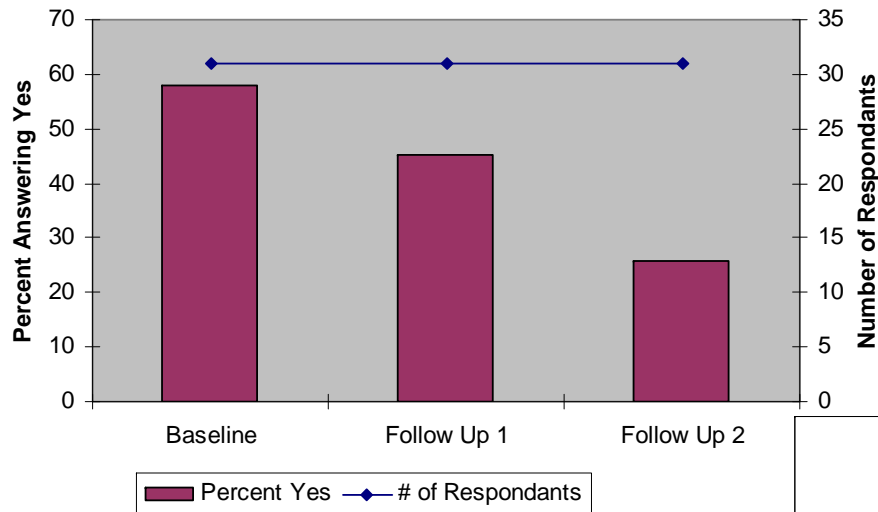


# No patients continue to rate their ability to manage their condition as poor or very poor after 2 program follow ups!

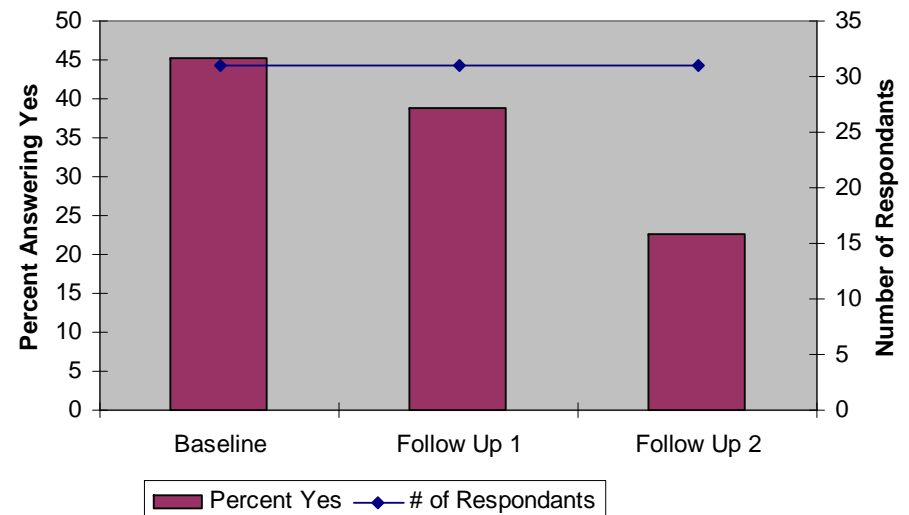


# Reduced depression symptoms

**Q52 - During the past month have you often been bothered by feeling down, depressed, or hopeless?**

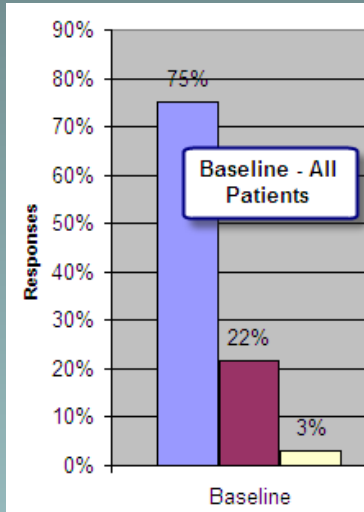
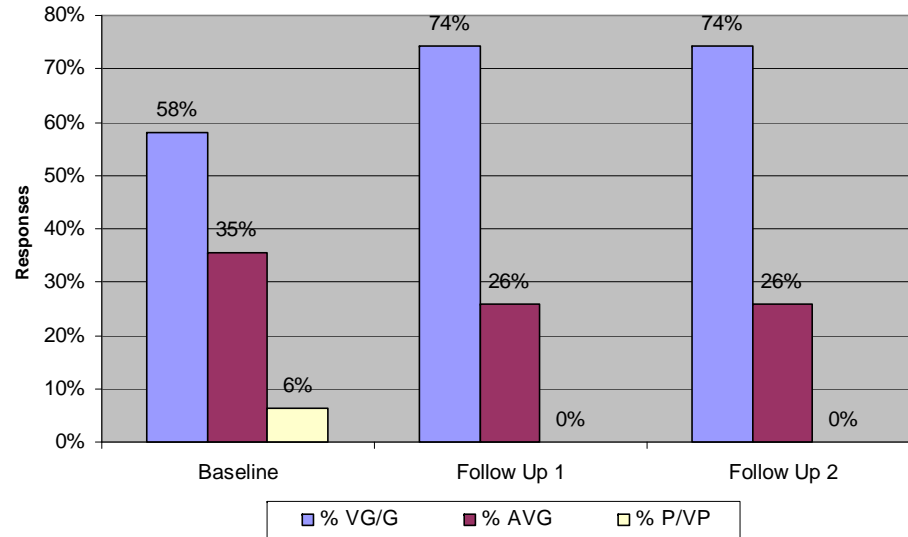


**Q53 - During the past month have you often been bothered by little interest or pleasure in doing things?**



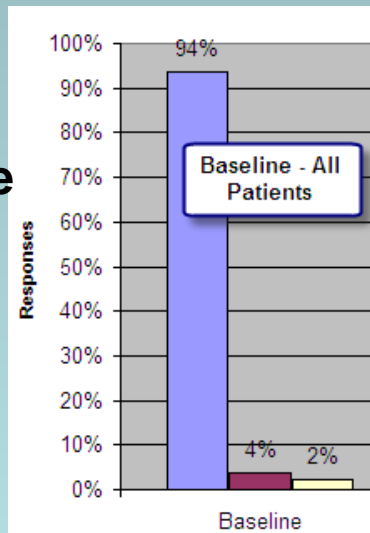
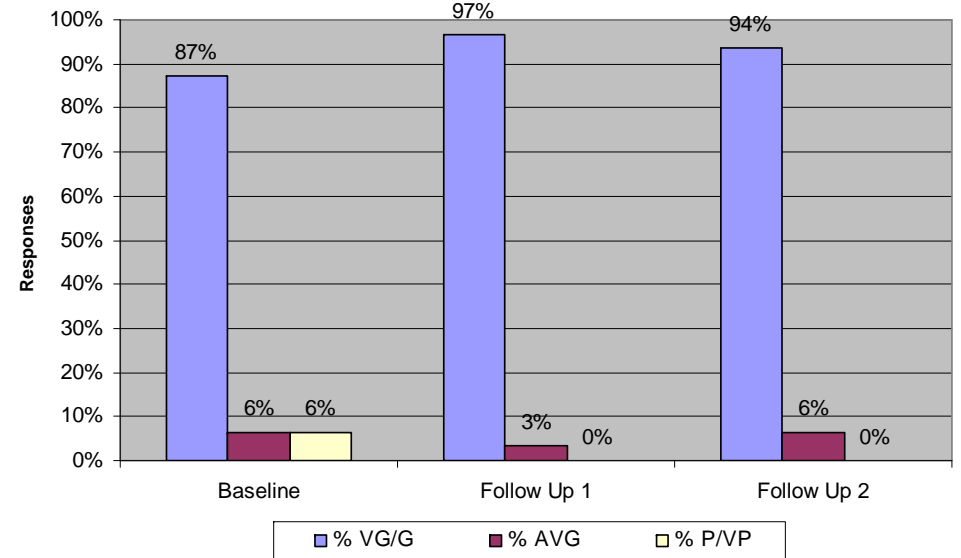
**Significantly improved Medication knowledge**

**Q28 - How would you rate your overall knowledge about your medications, its use and side-effects?**



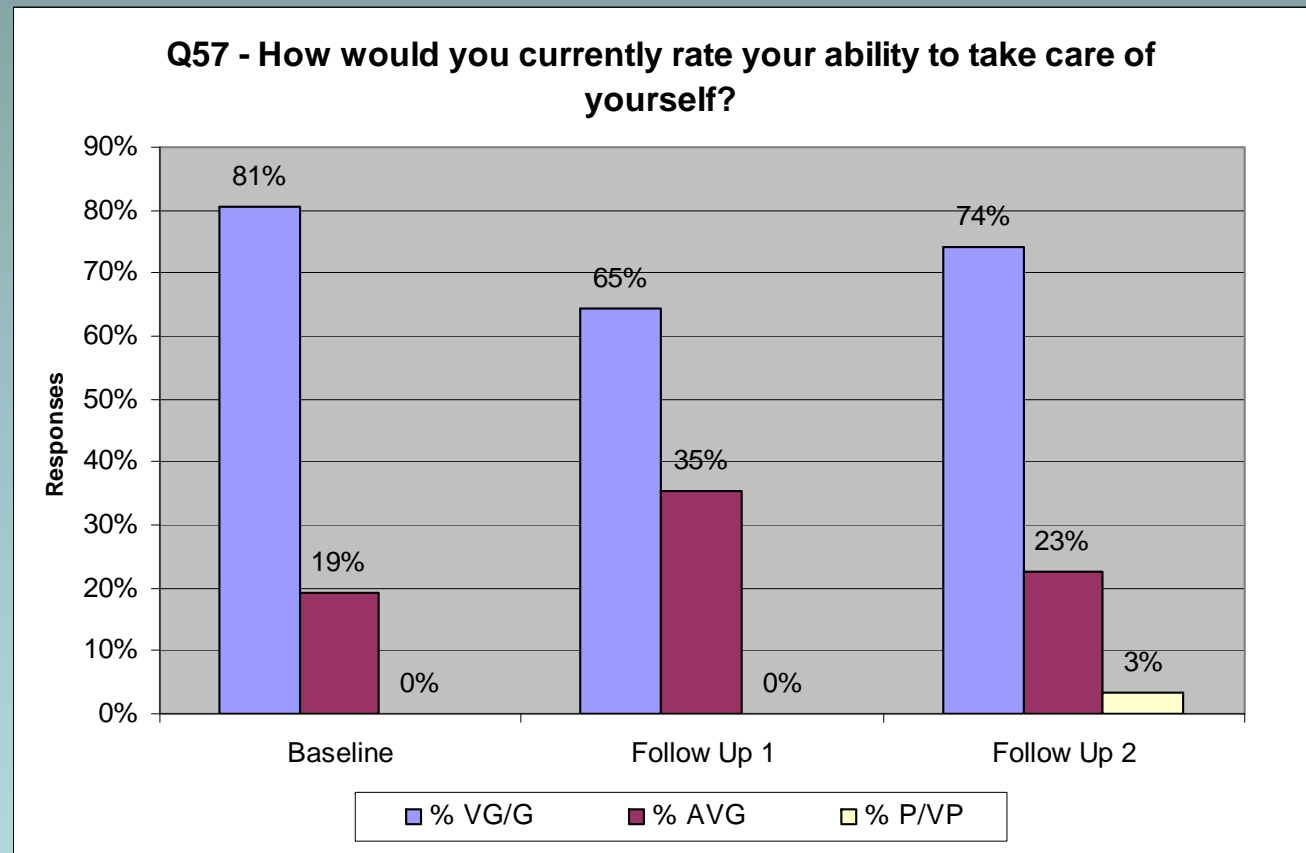
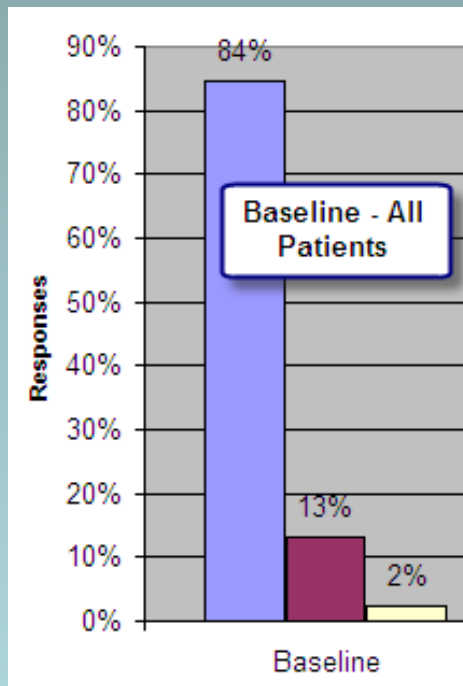
**Significantly improved Medication compliance**

**Q29 - How good are you at taking your medication as prescribed?**



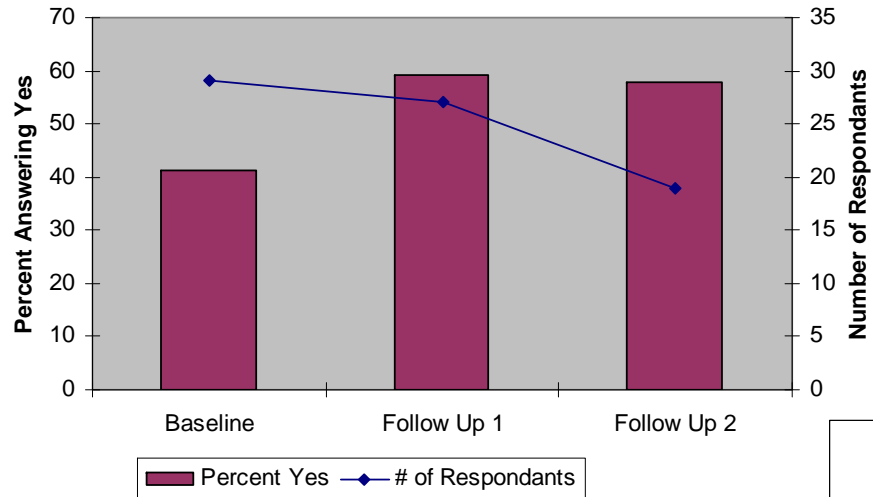
# Confidence to take care of yourself....

Increasing knowledge demonstrated earlier may be confounder.



# Improved health promotion / disease prevention

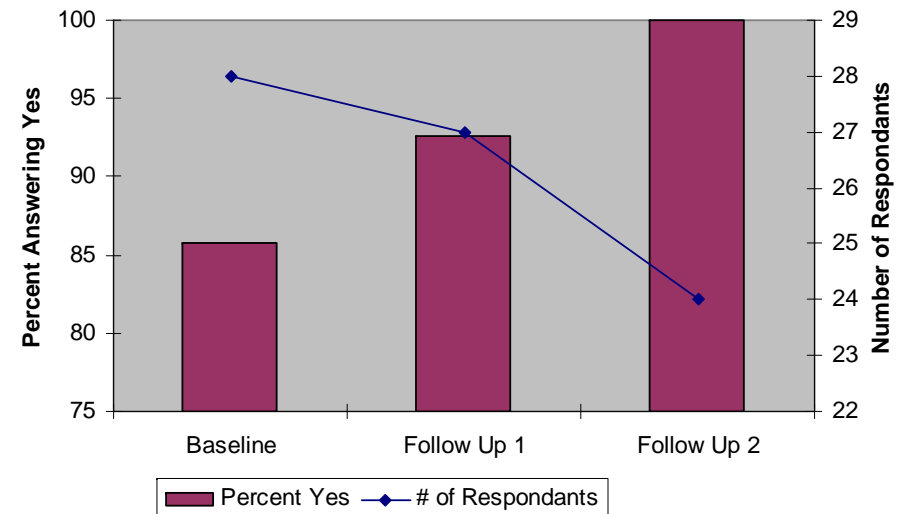
Q66 - Have you had a bone density scan/assessment in the last 12 months?



Bone density

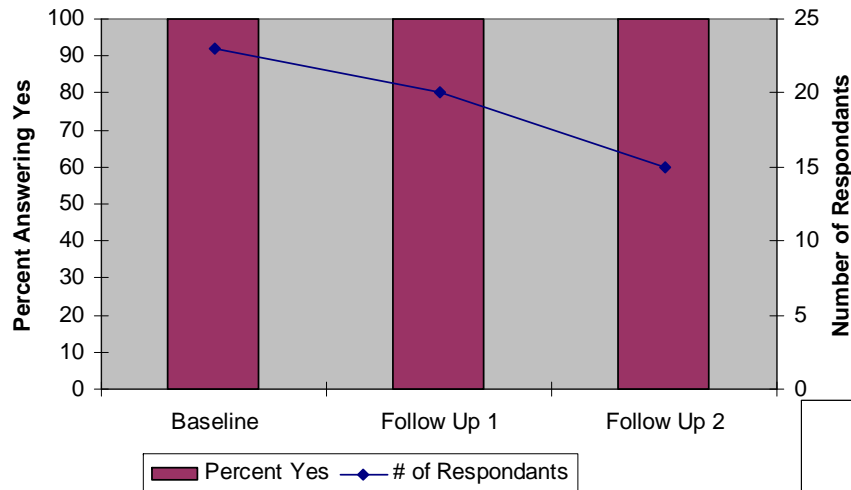
HTN screening

Q73 - Have you had your BP checked in the last 3 months?



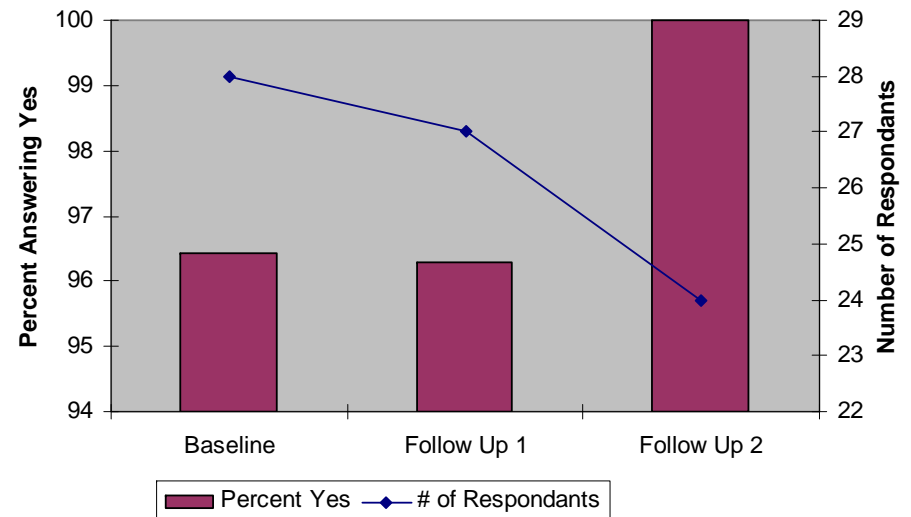
# Improved health promotion / disease prevention

**Q93 - Have you had a blood count (CBC) in the last 3 months?**



CBC

**Q95 - Have you had your liver function test (LFT's) in the last 3 months?**



LFT

# Program Impact....

<b>PROGRAM GOAL</b>	<b>OUTCOME</b>
Achieve and maintain remission, and manage relapses	(+)
Reduce patient's risks from prolonged or high dose steroid use	(+), need more data
Decrease the number of adverse drug events (ADE's)	(+), need more data
Improve patient's oral hygiene, and improve early identification of nasal, laryngeal and pharyngeal disease	(+)
Reduce occurrence or magnitude of secondary infection	(+)
Detect and improve psychosocial disorder	(+)
Improve quality of life and promote healthy lifestyle	(+)

# Program Limitations / Directions....

- **Enrollment: need to enhance**
  - IPPF Registry launch with patient-driven web-portal enrolled 400 patients in 4 months
    - CHR has developed and is currently testing patient-driven web portal for enrollment and data capture
    - IPPF will optimize “value attraction” from registry to HMP, and explore data sharing strategies
- **Strategies to assess program impact versus other factors that can influence outcomes over time**
  - In progress.
  - Will need to disseminate findings / knowledge
  - Assess value in improved care and reduced costs

the IPPF Community



Where YOU have a say

[www.pemphigus.org](http://www.pemphigus.org)

ARE YOU ENROLLED?  
YOU SHOULD BE!

**HMP**  
Health Management Program

CLICK HERE  
FOR MORE  
INFORMATION



Award winning  
Communications



Partners

The IPPF thanks our many partners for their support and dedication.

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FOR OUR REGISTRY**  
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PEMPHIGOID**  
from the bench to the bedside

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