

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

Accelerate Product Launch and Profitability by Building Regulatory Requirements into Distribution

August 24-26, 2009 | Ameristar Casino | St. Louis, MO

Conference Presentation



International Pemphigus and
Pemphigoid Foundation



Health Management Program

- Patient Advocacy Group – Specialty Pharmacy Partnership
- Ultra-orphan illnesses with inadequate public, professional, research and 3rd party awareness and support

Connecting
the community

**Patients
&
Families**



**Advocacy
&
Discovery**

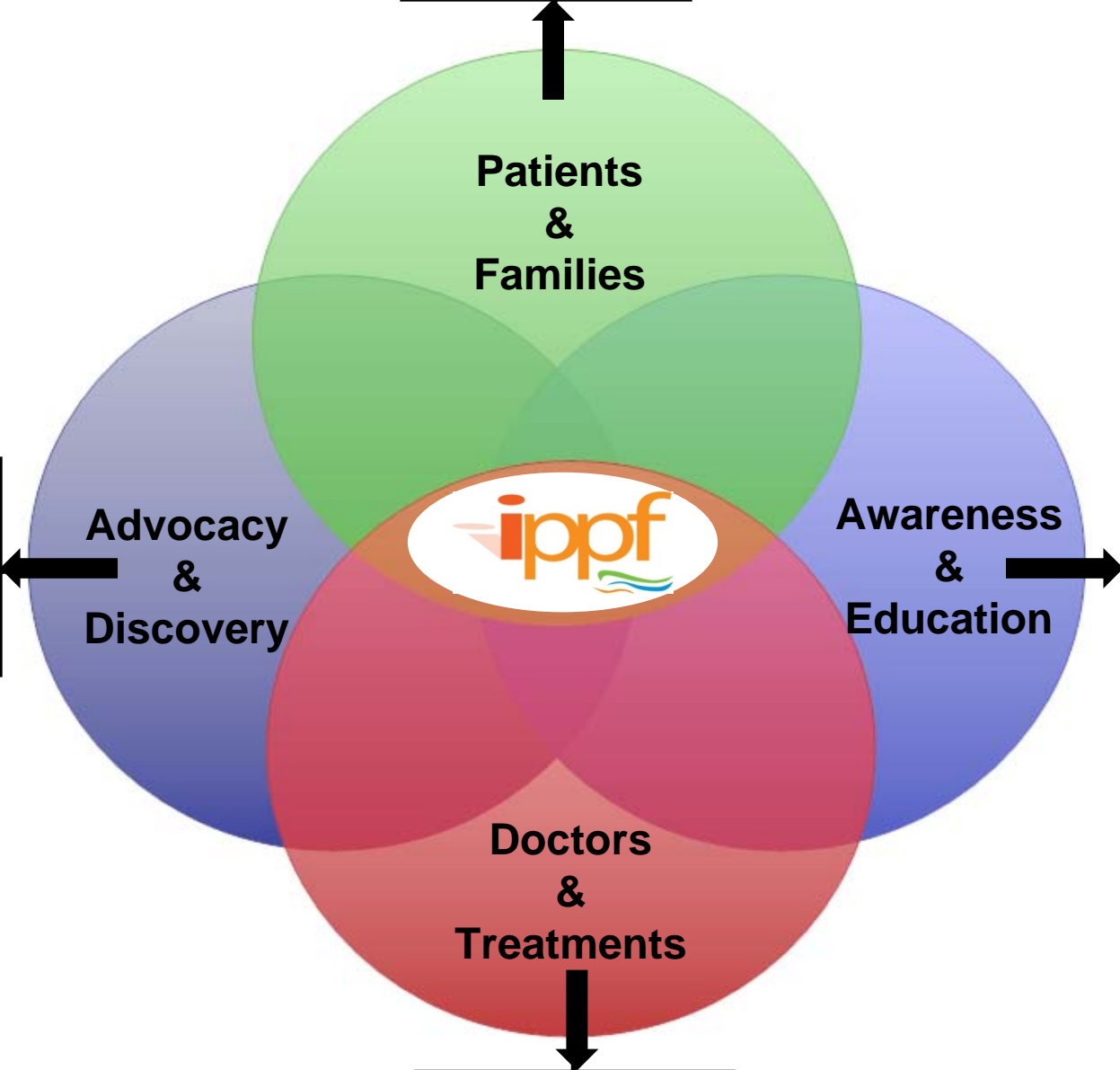
**Awareness
&
Education**

**Doctors
&
Treatments**

Referral, Access,
Best of Care

- Policy
- Research
- Pharma
- Technology

Public / Patient
Professional





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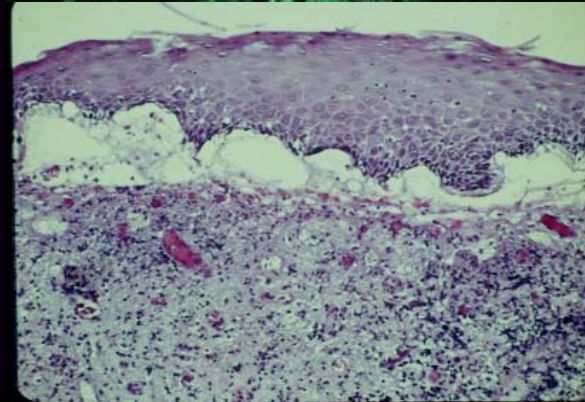
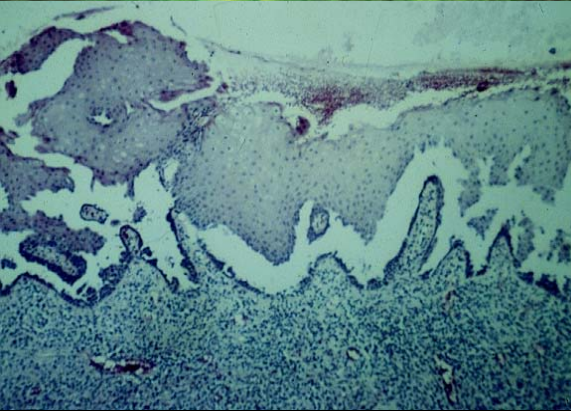
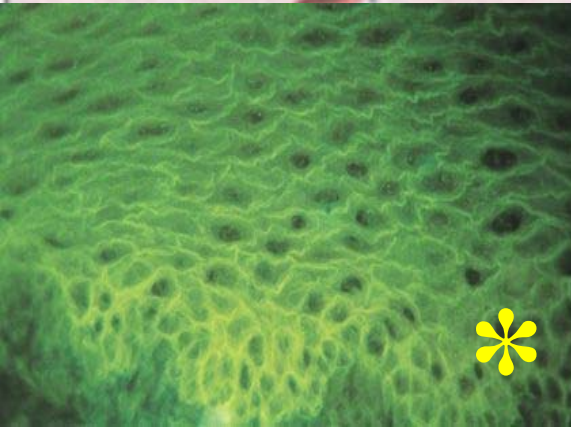
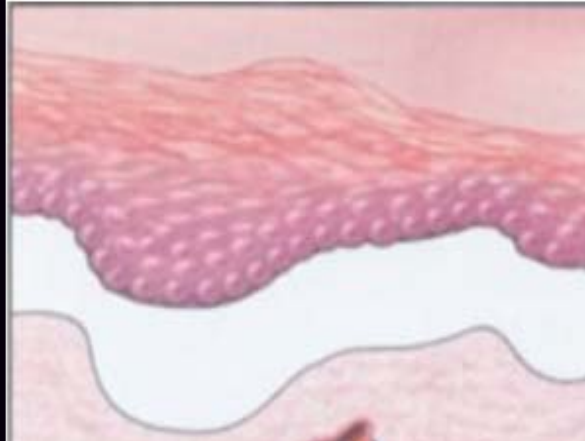
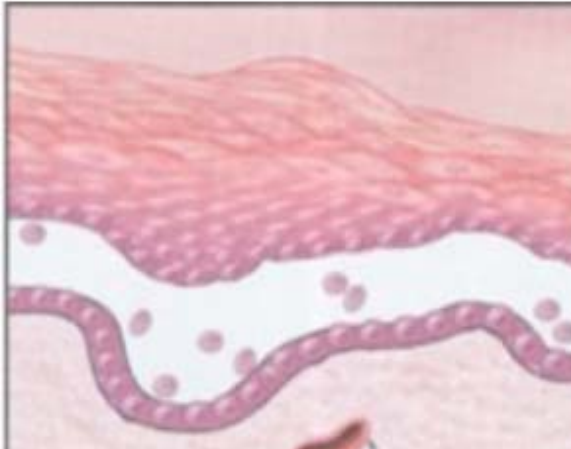
Health Management Program

- Patient education
- Disease data
- Treatment and outcomes data
- Improved compliance, disease control and quality of life
- Reduced complication / adverse events

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





pemphigus

pemphigoid



Treatments

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

Living with pemphigus and pemphigoid

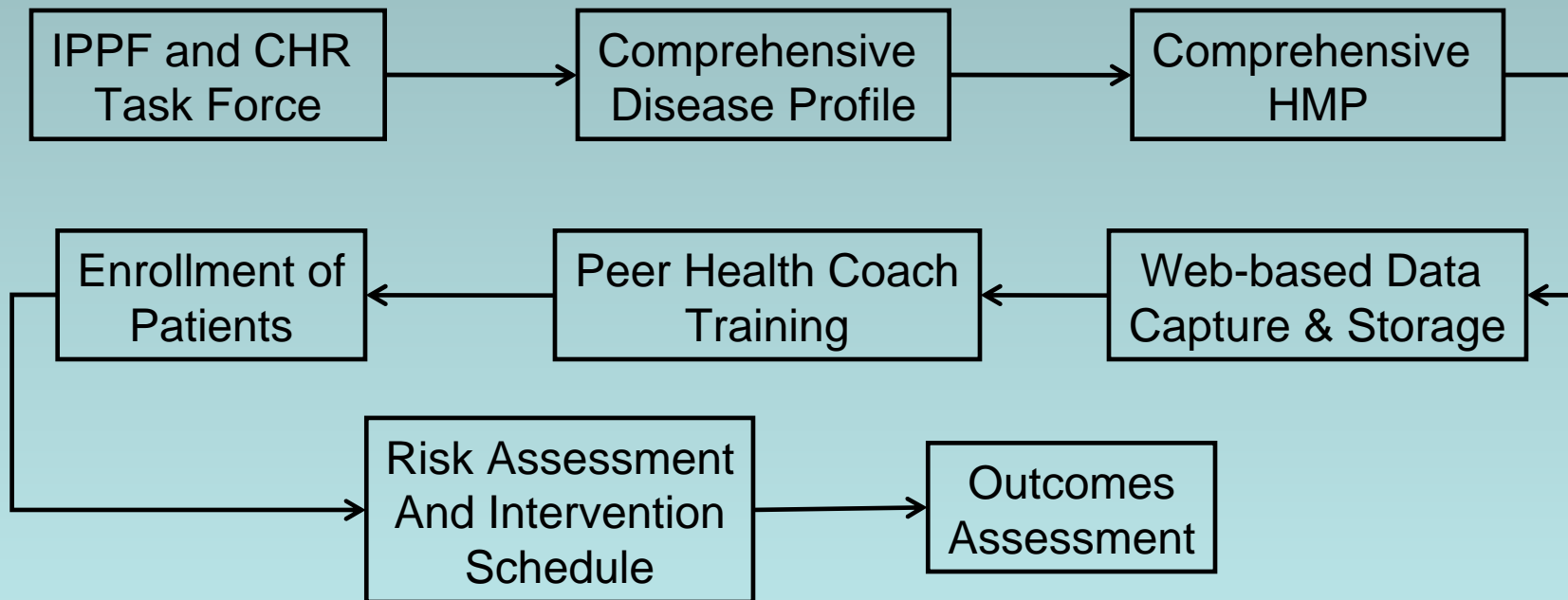
- Pain
- Lesions / wounds
- Infection
- Fear
- Anxiety, Depression
- Disability
- Appearance
- Medications
 - Costs & Access
 - Side Effects
 - Complications
 - Chronicity
 - Diabetes, hypertension, osteoporosis, adrenal suppression, immune suppression, marrow suppression, infection, GI, hepatic and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



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Health Management Program



- **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.

IPPF-CHR HMP Data

	Active	Inactive
pemphigus vulgaris	61	5
pemphigus foliaceus	11	2
pemphigoid	23	3
Total	95	10

- Mean age: 55 \pm 14.8 min: 22, max: 84 years
- 75F (71%) 20M (29%)
- Living in 36 states

IPPF-CHR HMP Data

- 14/105 patients had poor disease control (13%)
(5+ on 1-10 scale where 1=good and 10=bad)

5/10	6/10	7/10	8/10
4	4	4	2

Percent of active patients answering “yes” to these questions:	0 mg Pred	1-10mg Pred	>10mg Pred
Are you on adjuvants?	11%	39%	47%
Are you taking a Calcium Supplement?	84%	87%	68%
Are you taking an immunosuppressive?	42%	70%	56%
Are you taking any anti-inflammatories?	24%	22%	24%
Are you taking any Biologics?	13%	4%	9%
Are you taking any other RX meds at this time? (not for your primary condition)	84%	74%	68%
Are you taking medicines to prevent bone loss?	29%	35%	38%
Are you taking OTC/alternative meds at this time?	76%	78%	65%
Are you taking other therapy for this condition such as IVIG or plasmapheresis?	11%	4%	18%
Total n:	38	23	34

	Disease Activity	
Class Type	<p>"On a scale of 1 - 10, 1 = very controlled, 10 = out of control, how do you feel about your disease activity?" If answer ≥ 5, then high activity</p>	<p>"On a scale of 1 - 10, 1 = very controlled, 10 = out of control, how do you feel about your disease activity?" If answer < 5, then low activity</p>
Class 1 - Pred Therapy < 10 mg/day	Quarterly for first year, then every 6 months	Annually
Class 2 – Pred Therapy ≥ 10 mg/day	As needed, and at least monthly	Quarterly

IPPF-CHR HMP Data

Have you had a bone density scan/assessment in the last 12 months? 61% yes

Have you had an initial bone density scan/assessment
(prior to or within 3 months of starting prednisone)? 49% yes

- 50/105 (47%) active patients improved disease control while enrolled
- Of the 95 active patients, 57 were taking prednisone, and 39 (68%) reduced prednisone use while enrolled

Extending Impact

- 2009 YTD 100,000 page views of website
- Over 3,000 unique visitors to website ea month
- >400 new contacts to IPPF / year
- ~1,000 new members to our “web” community / yr
- 2009 YTD: 17,000 e-blasts to constituents on P-P info
- Enhanced patient management systems for delivering information, referrals, opportunities
- Deeper penetration into the illness community