CARING FOR CARCINOID FOUNDATION

Dedicated to discovering a cure for carcinoid cancer

Considerations Regarding Food and Drug Administration Review and Regulation of Articles for the Treatment of Rare Diseases; Public Hearing

Docket No. FDA-2010-N-0218 – June 29/30, 2010

Caring for Carcinoid Foundation - Our role in bringing treatments to patients

- **Mission**
  - **Discover a cure** for carcinoid cancer and related neuroendocrine tumors
  - **Eliminate the suffering** of patients, families, and caregivers affected by these cancers
- **History**
  - Founded in 2005 by metastatic carcinoid cancer patient, Nancy Lindholm
  - To date, we have funded over **$6 million** in cutting-edge research
- It is the **ONLY** foundation aggressively pursuing a cure for carcinoid cancer and related neuroendocrine tumors through funding research across **genomics, basic science and translational science**

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Carcinoid cancer and neuroendocrine tumors – definition and epidemiology

- Discovered over 100 years ago
- **Over 100,000 patients** in the US
- **No FDA approved cure**
- Very limited FDA approved treatment modalities
- No clear standards of care
- These are rare cancers (1 in 3,000 people) – incidence is increasing for unknown reasons
- Fatal and incurable unless caught early, which they rarely are
- These cancers can secrete hormones and cause “carcinoid syndrome” and other debilitating syndromes and side-effects
- For hope, patients turn to radical surgery, clinical trials or other investigational therapies

Barriers to development – (1) Bench

- **At the Bench** –
  - No deep understanding of neuroendocrine tumors
  - No cell lines
  - No animal models
  - No natural history data
  - Variable clinical course
  - No proven surrogate endpoints

- Without these basic resources and data sets, clinicians use guess work in clinical trial design.

Barriers to development – (2) Clinical trials

- **During Trials / Recruitment –**
  - Small patient population
  - Pre-treated patient population
  - Disperse patient population
  - Physician bottleneck
  - Lack of information on cutting-edge treatments
  - Lack of surrogate endpoints

- Rare, indolent diseases may require decades of study before causality can be established.

Barriers to development – (3) Investment risk-reward

- **Investors need**–
  - Incentives: e.g. Orphan Drug Act
  - Clarity
  - Consistency & reliability
- Disease-focused non-profits like Caring for Carcinoid Foundation invest in overcoming barriers to development.
- Caring for Carcinoid has invested over $6 million; in other words, $60/ patient to date.

Current Considerations for Patients

- Investigational radiopeptide therapy
- Radical surgery
- Enroll in clinical trials
- Investigational therapies
- Best option may be NO intervention
- High socio-economic impact for patients
- Significant impact of this disease on the U.S. health care system

Suggestions for the FDA:

- FDA Orphan Products Grants program—increase funding
- Consistent, flexible, and predictable
- Focus on patient needs and perspectives
- Pay attention to rare diseases
- Collaborate with NIH to focus on rare diseases
- Focus more effort, attention and funds on rare diseases. It matters to 30M people.