Unmet Needs in Gastroparesis

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Oley 2021
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- Main funding: NIH GpCRC
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- Consultant: Censa, Nuvaira, Takeda
- Speaker: Takeda, Medtronic
- Reviewer: UpToDate
- Writer: Med Study
- GES editor: Neuromodulation, Wikistim
- ADEPT-GI: IP on bioelectric therapies/ANS-ENS profiling

Aylin Tansel
- None
Survey

• Questions are on Zoom
• Confidential, no identifying information
• Participation is optional
• Goal is to assess needs
  • Hope to share results of survey
Survey
Patient and families

• Information about illness online
• Providers willing to see
• Access to new therapies
• Home health care issues
• Nutritional needs
Patient and families

- Inability to lead a normal life
- Reduced quality of life
- Reduced ability to work
- Need for more effective therapies
- Gap between patient and caregiver perceptions
Providers

• Information about proper diagnosis
• Information about good therapies
• Ability to refer patients for care
• Support group contact information
Insurers

- Standardized Diagnosis
- Evidence based recommendations for Therapy
- Published outcome data
Industry

• Guidance for clinical trials
• Agreement on diagnosis
• Recommendations on therapy
• Reliable epidemiologic data
• Accurate data on numbers of patients
• Guidelines on disability
• Information on costs of illness, for diagnosis and for therapies
Key points to unmet medical need in relation to different stakeholders

**Medicine developers**
- Public health needs and incentives
- Predictability of the value of pipeline investment
- Value and quality-adjusted life-year (QALY) thresholds

**HTA Bodies**
- Unmet medical need is formally and/or informally incorporated on a national level
- Some or all elements of unmet medical need may be a part of national value frameworks
- Quantification of relative benefits and uncertainty is important

**Patients**
- Adequacy, accessibility and affordability of existing treatments
- Disease severity and burden
- Length of development process and waiting time for patients

**Regulators**
- Unmet medical need already formally incorporated
- Unmet medical need is currently a binary decision, no quantification
- Decision based on:
  - Proof of concept
  - Preliminary Evidence
  - Benefit/risk
- Ultimately patient perspectives should be applied to unmet medical need considerations

**Payers**
- Unmet medical need may be formally and/or formally incorporated on a national level
- Emphasis on well defined patient populations
- Size of relative benefits is important

Vreman RA et al Value in Health 2019
Gastroparesis unmet needs

• Gaps in current understanding of natural history of disease
• Define and achieve optimal treatment goals.
  • Improvement of quality of life
  • Minimization of adverse events
  • Reduction of extent of tissue damage
  • Reduction of hospitalization
  • Reduction of need for surgery
  • Reduction of need for intravenous support
Gastroparesis Points

• Early diagnosis and treatment are important
• Reduction in time to diagnosis
• Timely and effective interventions have the potential to improve outcomes
Achieve these goals

• Induce and maintain symptom remission
• Restoration of motility if possible
• Bridge the existing gap between current healthcare practice and optimal treatment strategies
• Accurate biomarkers to assist selection of the most appropriate therapies
• Treatments that improve the natural history
Patient focused goals

- PROs beyond pain/nausea/vomiting/bloating need to be defined and validated to provide comparable results across studies.
- Patients’ perspectives and expectations are poorly understood.
- Active involvement of patients in clinical decision making improves patient satisfaction and probably disease outcomes.
Unmet Patient Needs

• More than 500,000 de-identified and anonymous public comments that occurred in openly accessible digital media for patients living with one of ten chronic diseases from 2010 to 2018

• **Medical needs:** needs related to symptoms, treatment specifics, and curing the disease

• **Emotional needs:** needs related to knowledge and power to make informed decisions and prepare for daily life, how to manage the disease long term, and community engagement

NEJM Catalyst Jan 2019
Mapping unmet needs

1. Understanding medication side effects and impact on daily life
2. Coping with living with the condition
3. Understanding the disease and its causes
4. Identifying and mitigating symptoms
5. Connect with other patients to share
6. Managing disease progression
7. More and better treatment options
8. Understanding diagnostic tests, procedures, and results

Emotional need
Medical need

NEJM Catalyst Jan 2019 Quid Analysis
Thank you!