

# The Expanding Role of Patients and Advocacy Organizations in Advancing Research and Care

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Director of Research & Strategy, Contractor



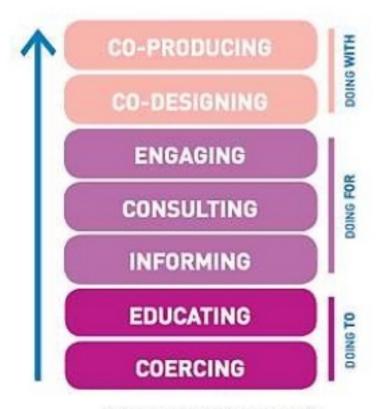
# Expansion from Passive Patients to Patients as Full Partners

### **A Continuum of Co Production**

**Partnership** 

Patient Voice/influence

**Passive Patient** 



Source: new economics foundation

## Savannah





### Lennox Gastaut Syndrome

**Age of onset** – 1-7 years of age

Seizure types – tonic (mostly nocturnal), atonic, myoclonic, atypical absence, generalized tonic clonic, focal

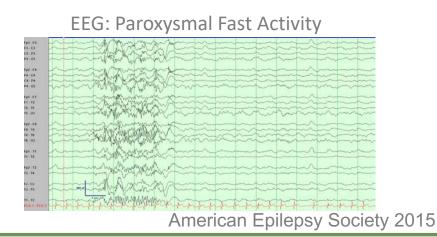
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**Common etiologies** – variety of etiologies, proceeded by infantile spasms in 9-40% of cases

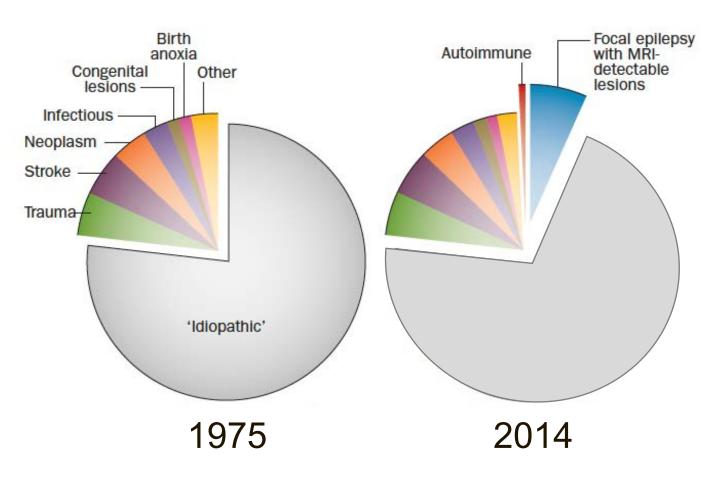
**Treatment** – felbamate, clobazam, rufinamide, topiramate, zonisamide, ketogenic diet, valproate, levetiracetam, VNS, corpus callosotomy, focal cortical resection (if there is a focus)

Prognosis – moderate to severe intellectual impairment, usually correlates with etiology and seizure control





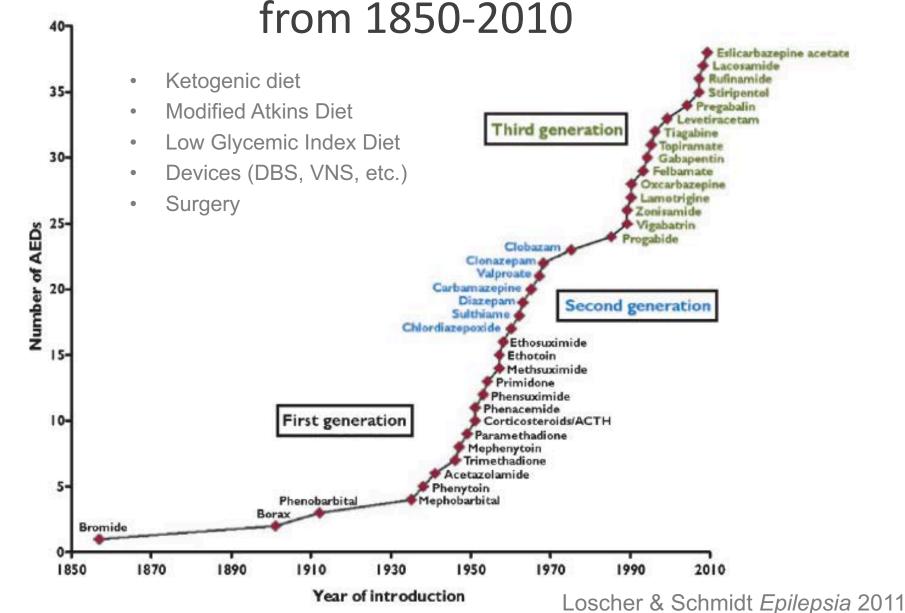
## Causes of Epilepsy



Hauser & Kurland 1975

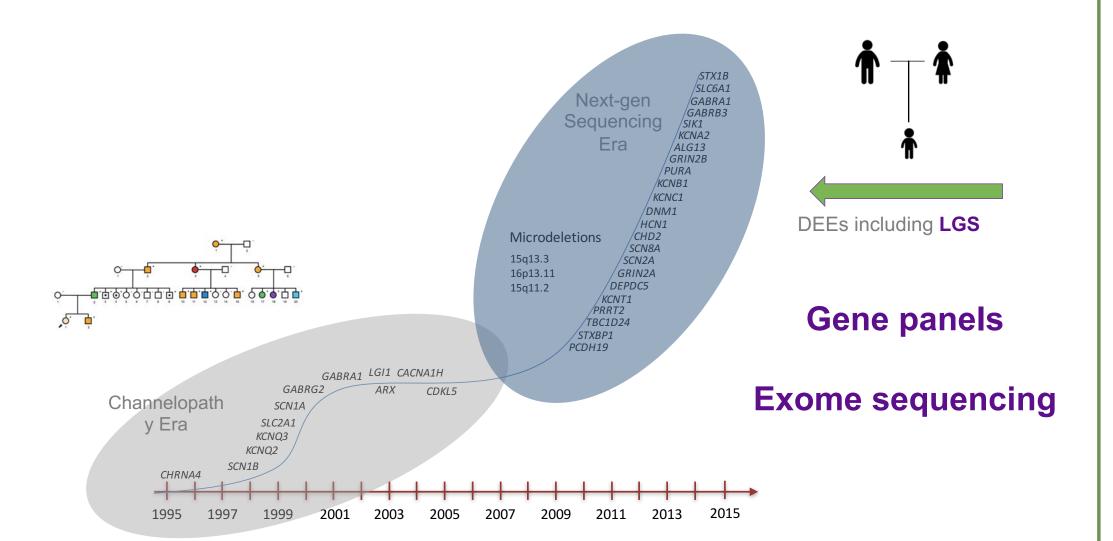
Thomas & Berkovic 2014

# ~40 approved epilepsy medications from 1850-2010

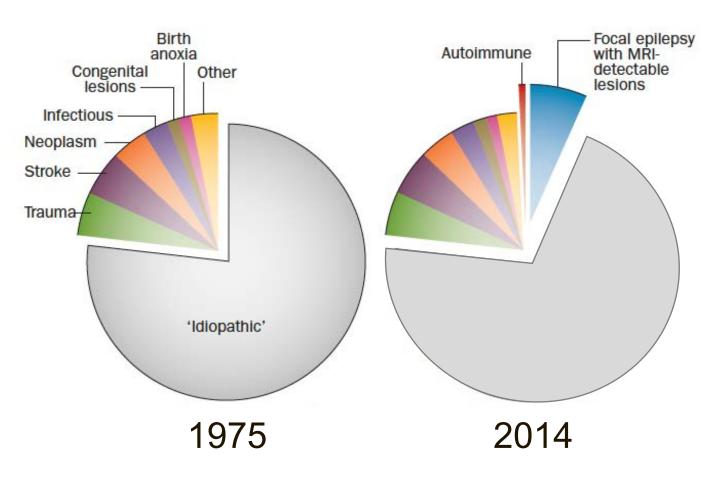




## Gene discovery in epilepsy



## Causes of Epilepsy



Hauser & Kurland 1975

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## Savannah's Odyssey 1995-2011

• # of Seizures: >40,000

• Diagnosis: 3 years

• # Neurologists: 7

• # Treatments: 26

• Cause: Unknown

• Hospitalizations: 15

• Surgeries: 5

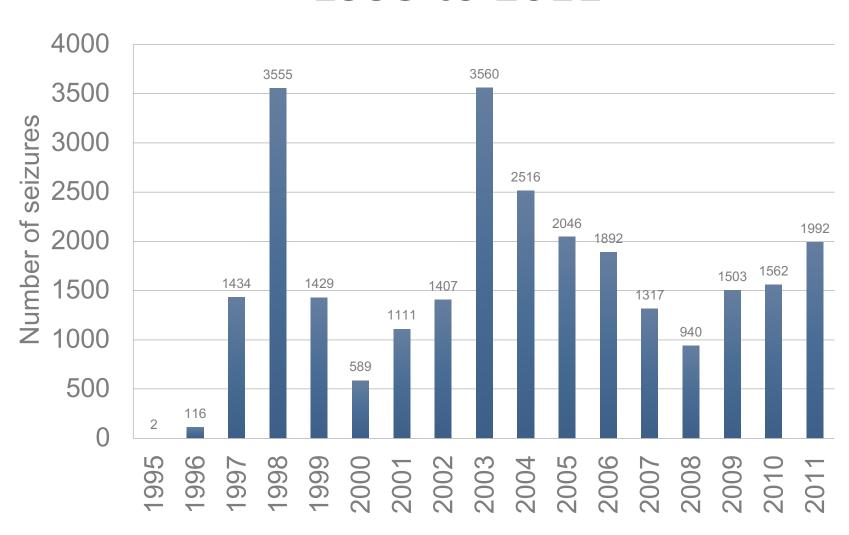
• Monthly drug cost: \$1,640

• Diastat use: \$183 / dose

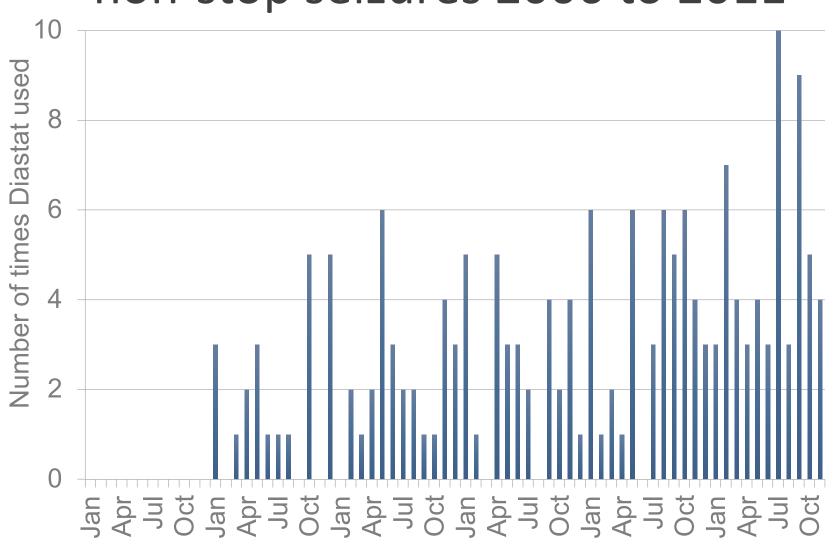
• Last stay: \$53,475



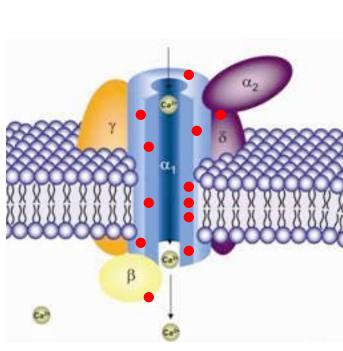
# Number of seizures per year 1995 to 2011



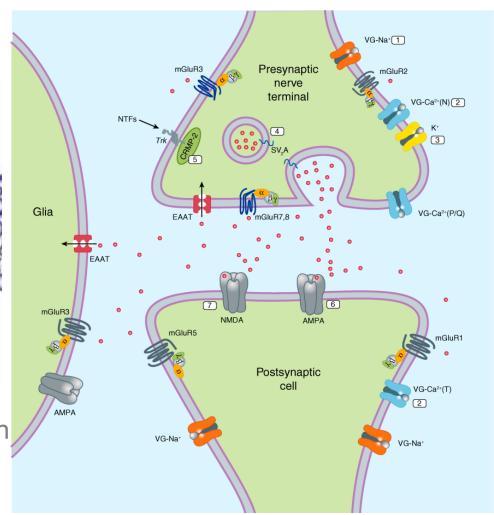
# Monthly emergency medication use for non-stop seizures 2006 to 2011



# Exome identified numerous high impact variants in calcium channel subunits



Approximate variant location

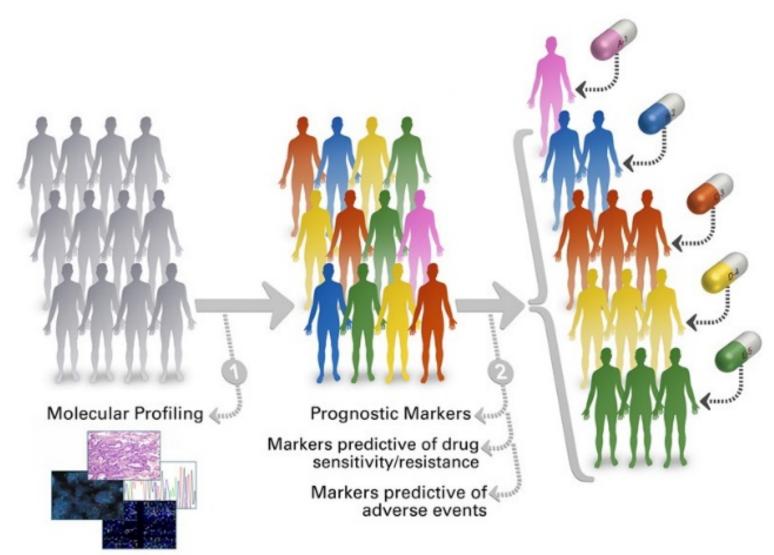


## Savannah Today





## Stratify Patients by Etiology



## Precision therapeutics – now

			Other Clinical
Gene	Syndrome	<b>Change in Management</b>	Implications
SCN1A	Dravet, Migrating epilepsy of infancy	Avoid sodium channel agents, Stiripentol	Monitor gait, Increased risk of premature death
SLC2A1	Glucose transporter deficiency (GLUT1)	Ketogenic diet	Monitor for movement disorder
HLA-A, HLA-B	Variants found in certain ethnic groups	Avoid Carbamazepine	-
PRRT2	Infantile convulsions	Consider Carbamazepine	Surveillance for other neurological issues
TSC1, TSC2	Tuberous sclerosis, Infantile spasms	Consider Vigabatrin, Rapamycin	Surveillance for tumors, non-neurological issues
ALDH7A1, PNPO	Severe early-onset epilepsy	Supplement with Pyridoxine (Vitamin B6)	-
PCDH19	Female epilepsy with intellectual disability	Avoid carbamazepine, consider Clobazam	-
POLG1	Mitochondrial epilepsy disorder	Fatal liver toxicity with Valproate	-

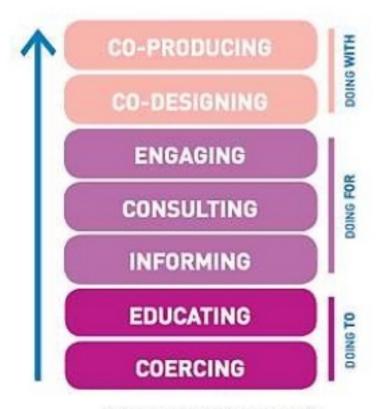
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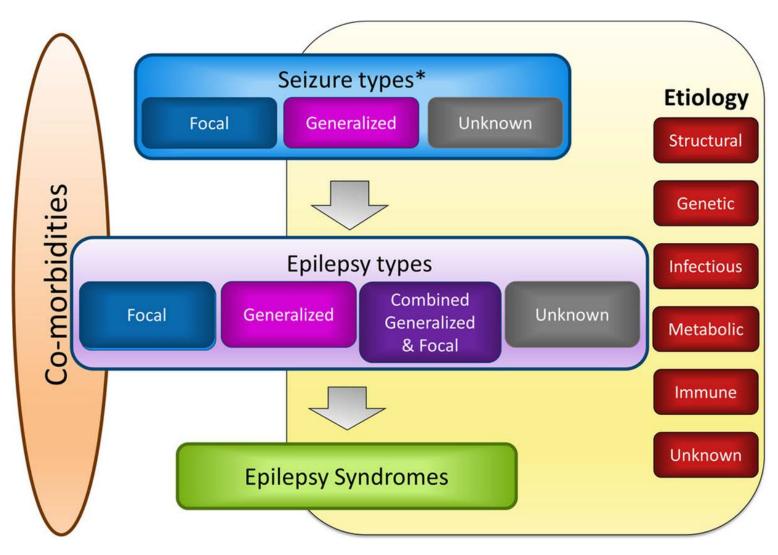


Source: new economics foundation

## From Science to Patient Experience



## ILAE Epilepsy Classification



Scheffer IE et al. Epilepsia 2017; 58:512-21

### Lennox Gastaut Syndrome

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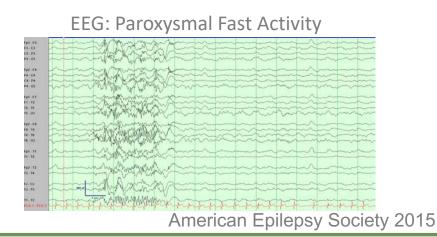
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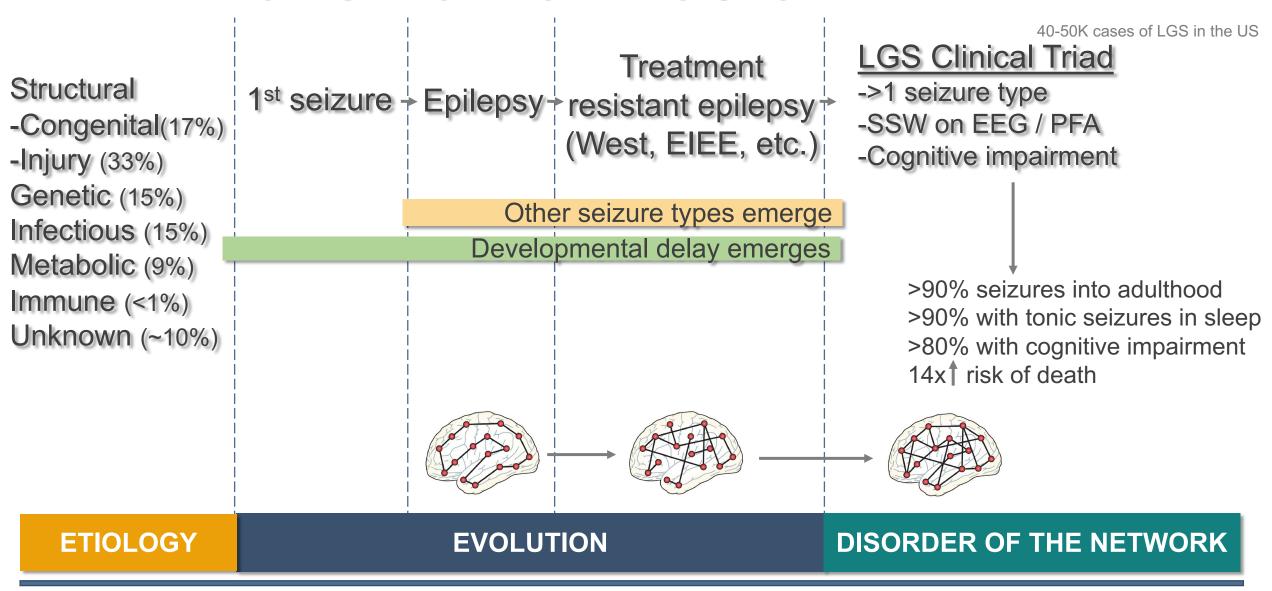
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### **EVOLUTION OF LGS OVER TIME**

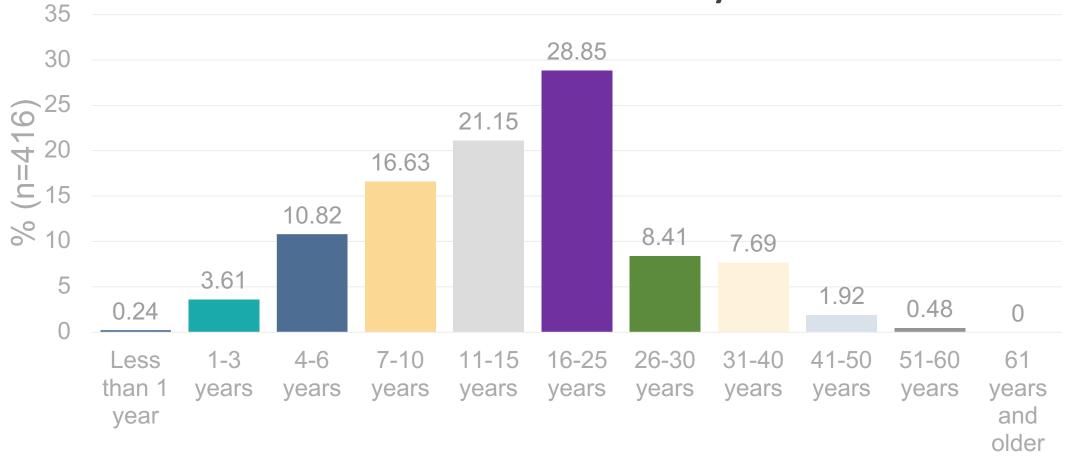


TIME

### LGS CHARACTERISTICS & CAREGIVER CONCERNS

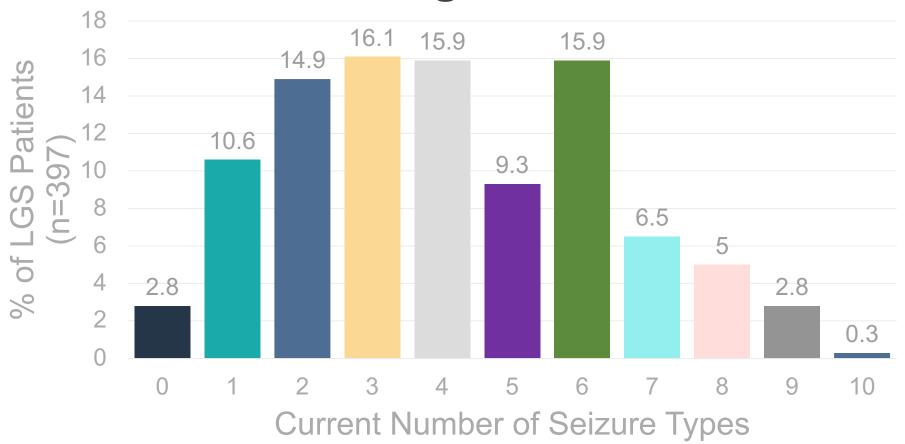
**LGS Foundation Caregiver Survey** 

What is the current age of the person with LGS that you care for?



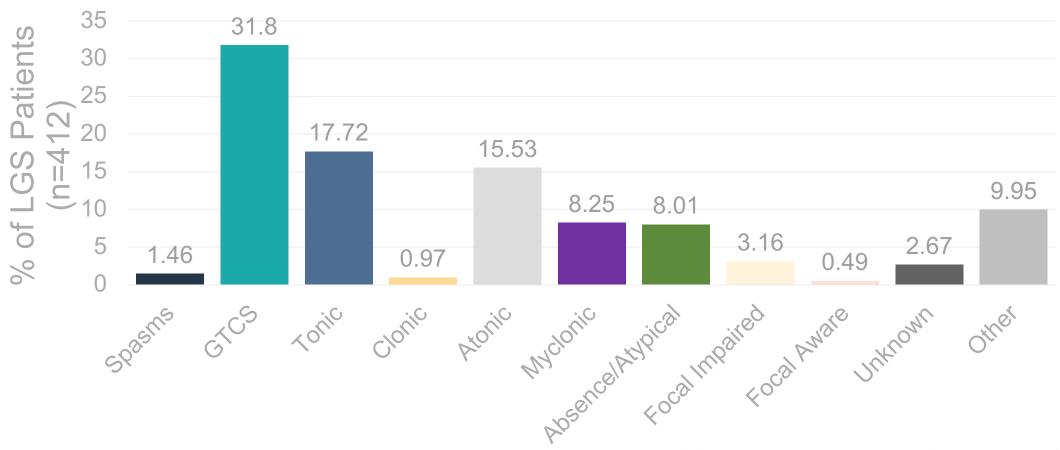
### LGS CHARACTERISTICS & CAREGIVER CONCERNS

# How many types of seizures is the person with LGS having AT THIS TIME?

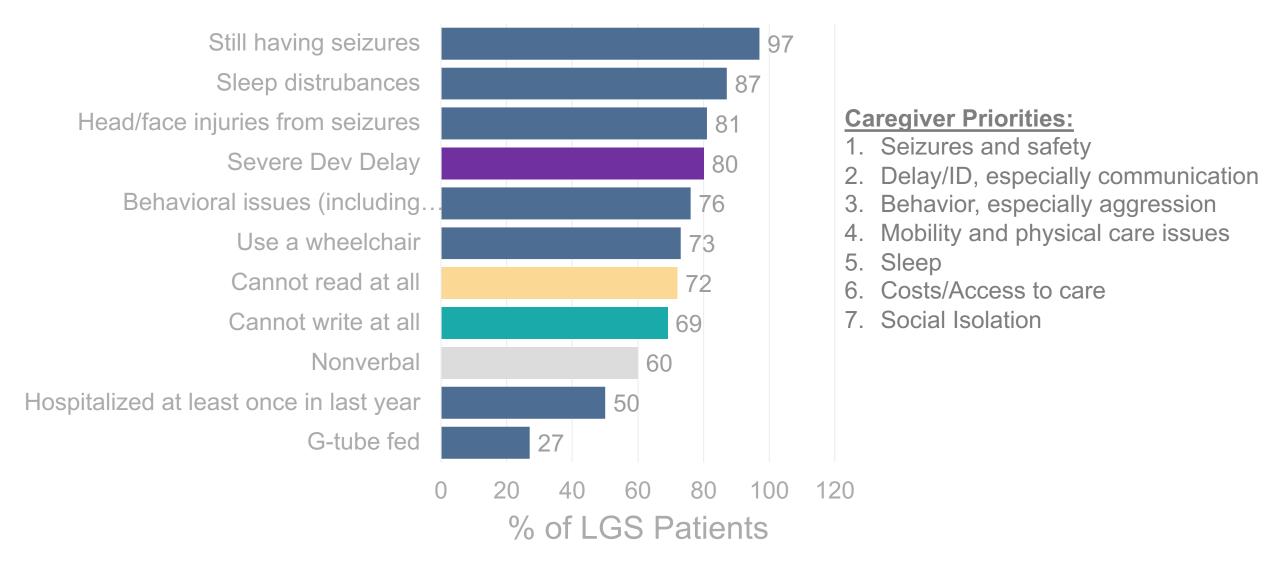


### LGS CHARACTERISTICS & CAREGIVER CONCERNS

What is the most problematic seizure type they are dealing with AT THIS TIME?



### **MAJOR ISSUES IN LGS REPORTED BY CAREGIVERS**



## LENNOX-GASTAUT MEDICAL HISTORY – REN SURVEY

### **Poor Seizure Control**



A majority of respondents had observed over 1,000 seizures in the past 6 months

14% have <u>never</u> sat up without help

24% have <u>never</u> walked

43% have <u>never</u> spoken a 2 word sentence



### **Co-occurring Conditions?**

- 33% have Autism
- 17% have ADD/ADHD
- 9% have Anxiety Disorder
- 5% have Depression

n = 200



## LENNOX-GASTAUT MEDICAL HISTORY – REN SURVEY

### On average

10-11 Different Anti-Seizure Medications Tried

### 

3 -4 Anti-Seizure Medications Currently Taking

### **Top 5 AEDs Currently Using**

- Clobazam (58%)
- Rufinamide (35%)
- Valproic Acid (33%)
- Levetiracetam (27%)
- Felbamate (26%)

### **Top 6 Discontinued AEDs**

- Topiramate (67%)
- Levetiracetam (61%)
- Valproic Acid (60%)
- Lamotrigine (59%)
- Zonisamide (55%)
- Clonazepam (52%)

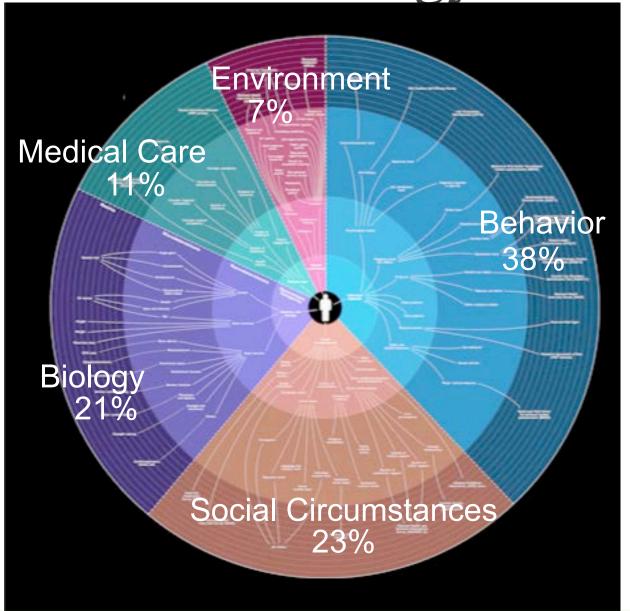
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## From Science to Patient Experience



Health is more than Biology & Medical Care

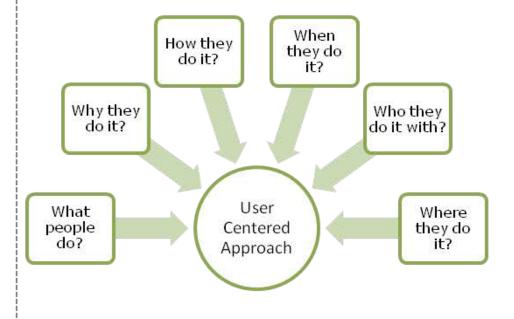


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## Change by Reframing Value



Product Product Conceptualization Architecting Product Product Development Construction **Product Release** 



Consumer-led research Specific product development

**User Centered Design** 

Marketing

**Product Development** 

Ethnographic Style Research

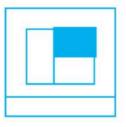


## Patient-Centered Outcomes

Patient & Family Costs

Quality & Applicability of Evidence

### What do Patients Value?



### PATIENT-CENTERED OUTCOMES

What are the clinical, functional, and quality of life benefits/drawbacks of different healthcare options to the patient? Quality of life -

Complexity of regimen

Efficacy & effectiveness -

Safety: side effects/complications



### PATIENT & FAMILY COSTS

What are the overall costs of different healthcare options to the patient and family? Medical out-of-pocket (OOP) costs

Non-medical costs to the patient & family

Future costs of care



### QUALITY & APPLICABILITY OF EVIDENCE

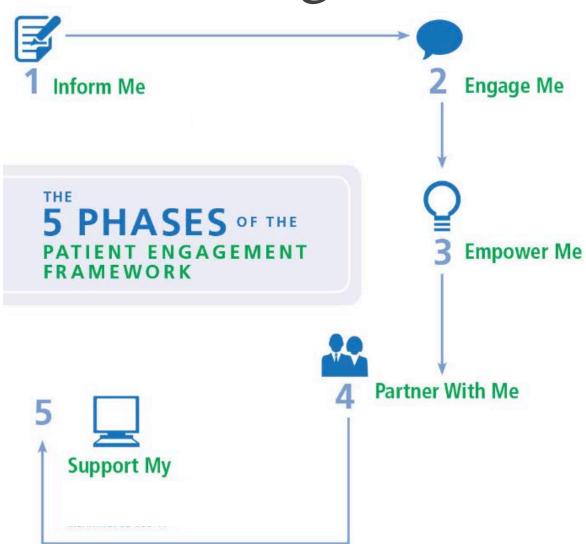
What level of confidence does the patient have that a healthcare option will have specific effects for them? Quality of evidence —

Consistency of evidence -

Differences in treatment effects -

### **FasterCures**

# Change by Partnering with Patients & Caregivers



HIMSS Foundation HIMSS.org





#### PATIENT ENGAGEMENT FRAMEWORK

RETAIN AND INTERACT

The Patient Engagement Framework is Iconsed under a Creative Commons Attribution-NonCommercial-NoDerlys 3.0 United States License.

REACH

EXTEND

SYNERGY AND

CREATE





#### Inform Me

#### Information and Way-Finding

- Maps and directions.
- Services directory
- Physician directory

#### e-Tools

- · Health encyclopedia
- Wellness guldance
- Prevention

#### Forms: Printable

- HIPAA
- Advance directives
- Insurance Informed consent.

#### Patient-Spedfic Education

- · Care plan
- Prescribed medication
- Tests
- Procedure/treatment

### Engage Me+ 9

#### Information and Way-Finding

Mobile

ATTRAC

AND

INFORM /

- · Nearest healthcare services
- Symptom checker

#### e-Tools

- Pregnancy tracking
   Option to share Fitness tracking
  - progress and health milestones
- Healthy eating tracking on social media

#### Interactive Forms: Online

- Patient profile
- Schedule a clinic
- Register or pay a bill appointment Email customer service • Refill a prescription

#### Patient-Specific Education

- Care instructions
- Reminders Medication
- · Preventive services · Follow-up

appointments

#### Patient Access: Records

- View electronic health record.
- Download electronic health record

### Empower Me + # + 9

#### Information, Way-Finding, and Quality

- Quality and safety reports on providers and healthcare organizations
- Patient ratings of providers, hospitals and other healthcare organizations

#### e-Tools

- · Care plan management
- Online nurse Secure messaging
- Virtual coaching

#### Integrated Forms: EHR

- Record correction requests
- Advance directives (scanned)

#### Patient-Specific Education

- Materials in Spanish
- Guides to understanding accountable care

#### Patient Access: Records

- Transmit patient record electronically
- · Copy the patient or a healthcare designee when sharing electronic record
- EHR integrated with patient PHR

#### Patient-Generated Data

- Care experience surveys
- Symptom assessments
- Self-management diaries
- Patient-generated data in EHR
- Questionnaires
- Pre-visit
- · Health history
- Demographics

#### Interoperable Records

- Integrated with health information exchange (HIE)
- E-referral coordination between providers
- Ambulatory and hospital records Integration
- Images and video in EHR.
- Commercial labs, radiology, medications

#### Partner With Me + # + + + 9

### Information, Way-Finding, and Analytics/Quality

- Patient-specific predictive modeling
- Patient-specific quality indicators
- **EFFICIENTLY** Patient accountability scores

#### e-Tools

- Wellness plan
- Advance care planning
- Coordination of care across systems

#### Integrated Forms: EHR

- Clinical trial records
- Immunization (public health)

#### Patient-Specific Education

 Materials in Spanish and the top 5 national languages

### Patient Access

tools

Devices

Advance

· Tele-medicine

treatment

Intolerances

Allergles

Values

for life-sustaining

· Condition-specific

self-management

- Publish and subscribe
- . Summary of care

#### Patient-Generated Data

- Shared decision
   Home monitoring maldng
- · Preferencesensitive care . Directives
- Informed choice/consent • Physician orders
- Adherence reporting
- \* Medications
- Self-care
- Wellness

#### · Preferences Interoperable Records

- Integrated with clinical trial records
- Integrated with public health reporting
- Integrated with claims and administrative data

#### Collaborative Care

- Acute
  - · Primary care Specialty
- Long-term post-acute care

очения принципальной принципальной выправления обращения общей различаю,

#### Information, Way-Finding, and Analytics/Quality

- Care comparison for providers, treatments, and medications
- · Costs Convenience
- · Quality

#### e-Visits and e-Tools

e-Visits as part of ongoing care

#### Integrated Forms: EHR.

(replaced by interoperable collaborative care records)

#### Patient-Spedfic Education

- · Care planning
- Chronic care self-management
- Reminders for daily care

#### Patient Access and Use

- Publish/subscribe for complete record
- Distribution of record among care team
- Patient-granted permissions
- Patient-set privacy controls

#### Care Team-Generated Data

- Shared care plans
   Team outcomes
- · Episodic

· End of life

- + Chronic
  - . Costs · Quality

· Adherence

Interoperable Records Integrated with long-term post-acute care records

#### Collaborative Care

- Chiropractic
- Alternative medicine
- Dentistry Home

#### Community Support Online community support forums and

- resources for all care team members Caregivers
  - . Clergy Counseling
- · Family · Friends
- · Services

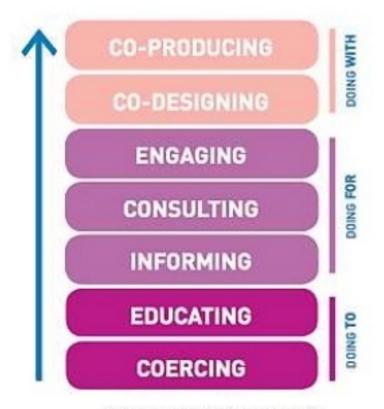
## Change by Co-Production & Co-Design

### A Continuum of Co Production

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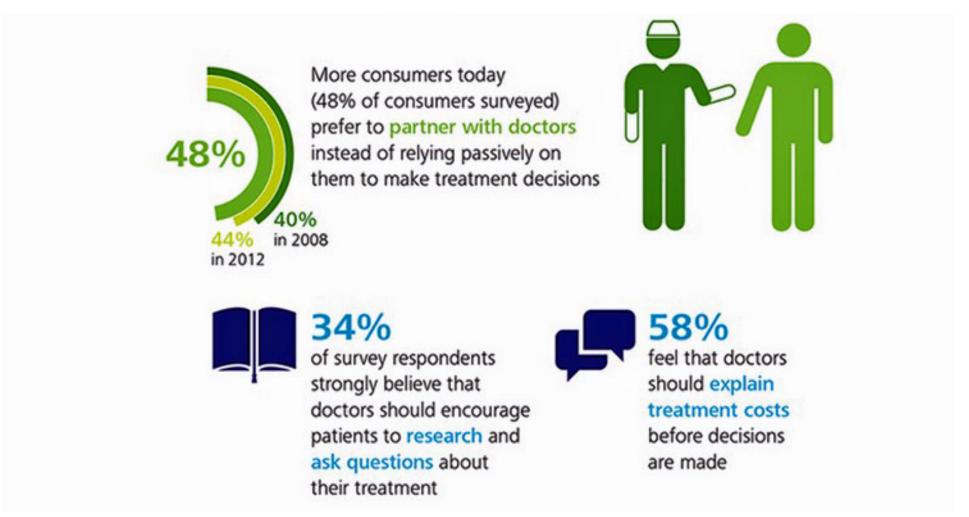
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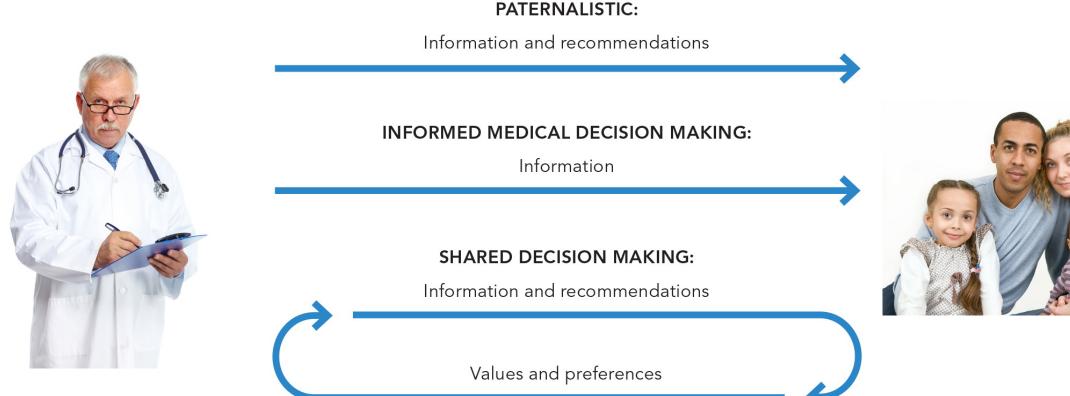


Source: new economics foundation

# More Patients Report Wanting to Partner With Doctors

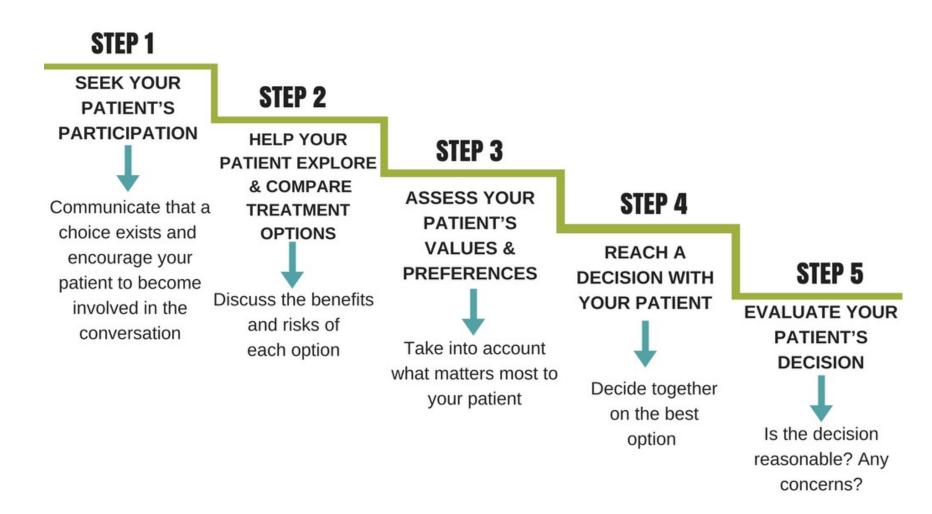


## **Shared Decision Making**



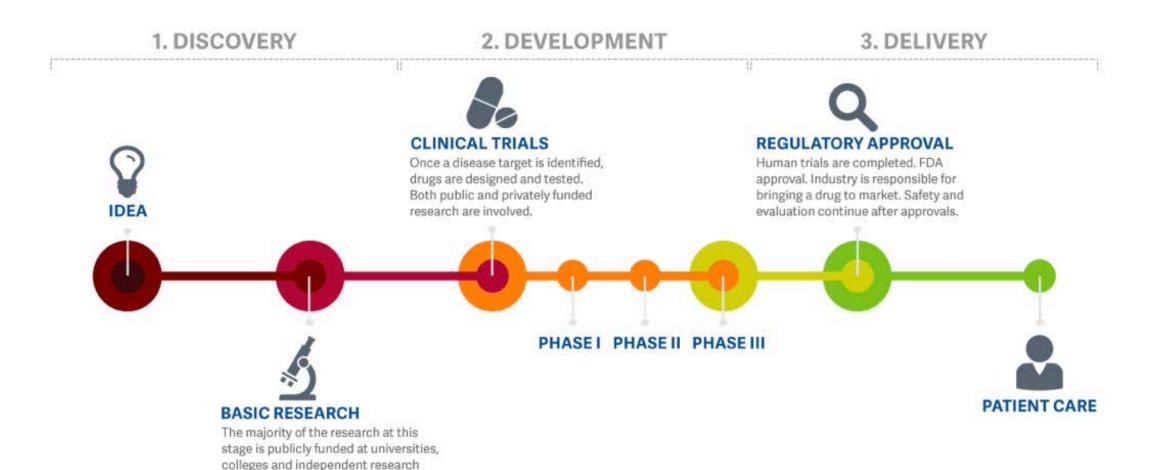


## **Essential Steps of Shared Decision Making**

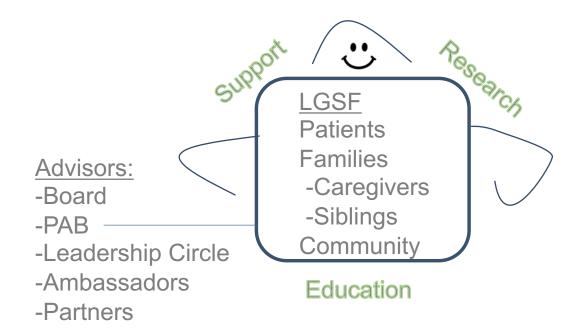


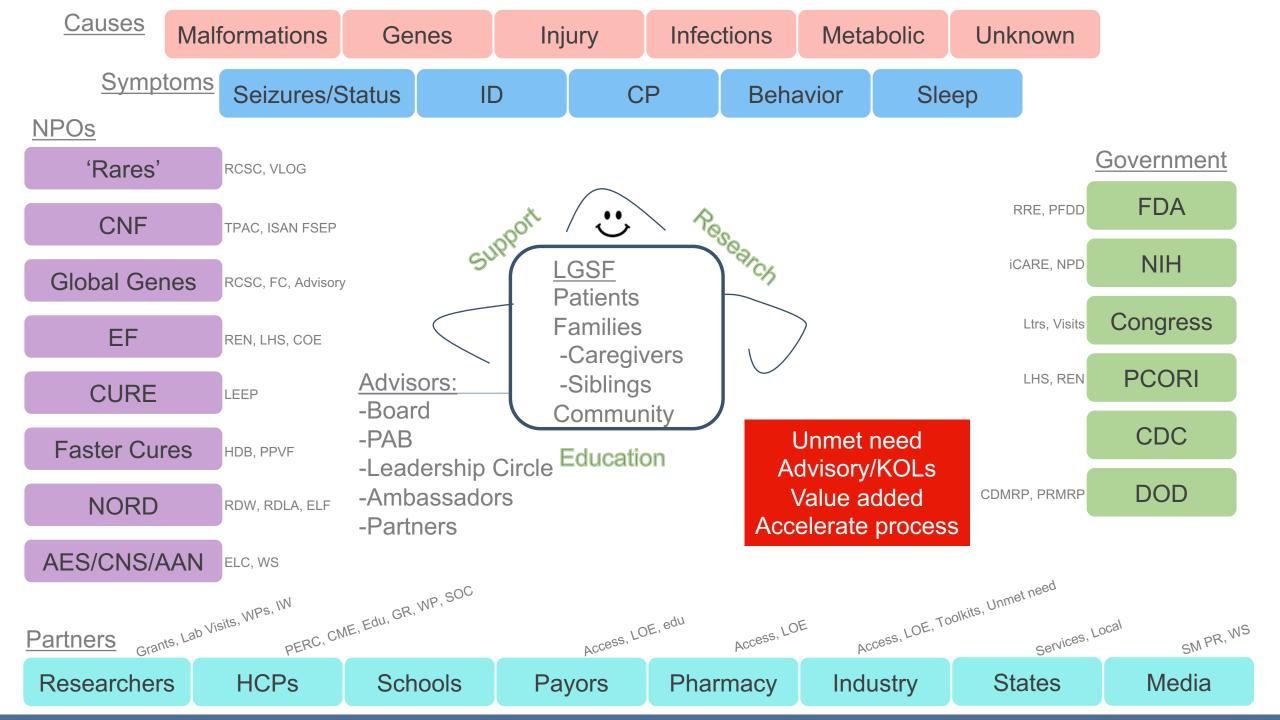
AHRQ website.

## New Ways to Engage Patients & Caregivers



institutions in every state.





# LGS FOUNDATION LENNOX-GASTAUT SYNDROME









