

HOW TO CONNECT WITH THE FDA, PATIENT RESOURCES, PIPELINE PROJECTS, PROGRESS OUT OF EUROPE AND AROUND THE GLOBE

**Resources and Innovations for Cushing's and Adrenal Insufficiency (AI) Patients**  
**CSRF Patient Summit**  
**Saturday, October 5, 2019**  
COMPILED BY AMY DAHM

**DRUGS in Progress – Pitched at World Drug Congress 2019**

- TBR- 065 and TBR-760, Tiburio, for rare endocrine disorders and non-functioning pituitary adenomas (NEPA). Tiburio is seeking patient input.
- ST-002, SteroTherapeutics, a DHEA-analog and anti-glucocorticoid that addresses non-alcoholic stenosis (NASH). Only drug under development that is a cortisol antagonist for Cushing's, anti-obesity properties, does not lower cortisol (mitigating accidental adrenal crises).
- Cibinetide, Araim Pharmaceuticals, promotes healing and repair of damage in the cardiovascular and neural cells. Although developed for sarcoidosis, could possibly enhance recovery of Cushing's patients.
- OSSGROW, ReGrow Biosciences, uses live cultured osteoblasts for regenerating hip bone tissue lost to vascular necrosis, a rare side effect in 5% of Cushing's patients.

**FDA PATIENT ADVOCACY**

New Director of Professional Affairs and Stakeholder Engagement, CDR Eleni Anagnostiadis, RPh, MPM

New FDA Patient Portal Launched Spring 2019: [www.fda.gov/RequesttoConnect](http://www.fda.gov/RequesttoConnect)

- Designed to give patient advocates a single point of entry, FDA routes to appropriate FDA office

Twitter account: @FDAPatientInfo

Phone Number: 1-888-INFO-FDA

**DATA**

*HVH Precision Analytics* – uses big data to find potential patient clusters by symptomology. HVH informs doctors, speeding up diagnosis. Has conducted initial Cushing's survey and is looking to partner up with Cushing's patients for further development. Contact info: Oodaye Shookla Oodaye.Shukla@hvhprecision.com

*Rare Disease Registry Program (RaDaR)* – online tool developed by NIH designed to help patient groups navigate and facilitate process of setting up registries <https://registries.ncats.nih.gov>

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*Rare Disease Registry Program (RaDaR)- continued...*

Launch of new, multi-year, patient-centered, comprehensive study about adrenal insufficiency under the auspices of the European Society of Endocrinology–

- Describe current service provision and patient education and identify gaps
- Develop a position statement on standards
- Interested? Contact Dr. Sofia Llahana Sofia.Llahana@city.ac.uk

### **TECHNOLOGY**

*Face Recognition* – hope for the future. Example: can scan a baby’s face and tell if the baby has a particular illness

*Cortisol Patch* – in the lab – Dr. Alberto Salles from Stanford and his team are developing a skin patch that can measure cortisol.

*Bristol Ultradian Study* – with a device that measures cortisol for 24 hours ultradian-study@bristol.ac.uk

### **PATIENT & CAREGIVER EDUCATION**

Steroid Education Group Meetings – discussed at World Alliance Pituitary Organization (WAPO) by Ms. Chris Yedinak

- Three hour lecture/ video/ demo workshop
- Pre- and Six-months post evaluation
- Focused on self-adjustment guidelines
- Resulted in reduced symptoms, reduced adrenal crises, lowered mortality

Launch of *Advanced Practice in Endocrinology Nursing* – edited by Dr. Sofia Llahana, endorsed by European Society of Endocrinology

- Full chapter on how to handle adrenal crisis
- Chapters available for download on [springer.com](http://springer.com)
- Note: CSRF’s own Dr. Meg Keil is one of the pediatric editors

Adrenals.eu & Adrenal App “everything about adrenal gland disorders”

- Covers adrenal-related illnesses in multiple languages
- Offers free tools in multiple languages, including European Emergency cards
- Adrenal App in English coming soon

Therapeutic Education in Adrenal and Pituitary Insufficiency (TPE): A Case Study, Lyon and Marseilles, French Endocrine Society

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Therapeutic Education in Adrenal and Pituitary Insufficiency (TPE): A Case Study, Lyon and Marseilles, French Endocrine Society – continued...

- Defined adrenal crisis as: the “mismatch between cortisol supply and demand, especially under conditions of physical and psychological stress.”
- Three steps: educational diagnosis, sessions, and program evaluation
- Recommendations:
  - Carry an emergency kit (cards, tablets, and injection kit)
  - Identify adrenal crisis: signs and risky situations
  - Know to adjust steroid doses
  - Know risk of overdose
  - Know how to administer HC by Solu-Cortef injections
  - Know how to use relevant resources of health care system & association
- Two flagship teams in Lyon and Marseilles from the national reference center led the way, while the French Endocrine Center developed two programs
- Six nurses and two endocrinologists built the workshops and selected patients
- Workshops: One-day format and trainers were 1-2 doctors or 1 nurse
- Eight sessions, including: Understanding my Adrenal Illness, Understanding and Managing my Adrenal Treatment, Preventing and Managing Adrenal Insufficiency, Know How to Do My Injection, Manage the Effects on a Daily Basis
- Included descriptive posters, Role Play/ Scenario Cards, Emergency Kit Models, Adrenal Crisis Booklets
- Patients re: advice re: high temps, jet lag over six hours, sports and training, pregnancy, every day situations. Doctors received advice re: endoscopy, general anesthesia for surgery, trauma, delivery, dental care

### **GUIDELINES**

Formation of an ECE Mission Statement and ECE Clinical Practice Guideline – as presented by Dr. Sofia Llahana

- Annual Patient Monitoring
- Patient education in adjusting steroids for sick days
- ALL PATIENTS SHOULD BE EQUIPPED WITH A STEROID EMERGENCY CARD AND STEROID SHOT

### **FREE MEDICAL- RELATED TRAVEL**

PALS – provides free airplane or shuttle travel to patients for medical-related travel and treatment

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### QOL RESEARCH

Dr. Elena Valassi from the Hospital Sant Pau, Barcelona, Spain

- Groundbreaking QOL data that validates patient feedback – scan QR code below to browse
- Recovery variables: age, gender, hypopituitarism, AI, brain structure
- Some symptoms can and do persist during remission: depression, impaired physical functioning, bodily pain, and poor general health
- 19% of patients have to stop work and 43% need some sort of reasonable accommodation
- Social and family life, sex life, relationships – not just work – impact patient's status

