

Rett Syndrome Europe Activity Report

2012 - 2013



RSE Board members (2013)



Martine Gaudy



Lorna Jaffa



Danijela Szili



Oliviero Dell'Oro



Thomas Bertrand



RSE aims

- **AIM 1 of the statutes:** To make Rett syndrome better known to the public, professionals, carers and those who are directly concerned in all European countries
- **AIM 2:** To improve the communication within the European Rett Community
- **AIM 3:** To promote as a representative European organisation, the interests of people with RTT and families
- **AIM 4:** To Expand RSE to all European Countries and to assist, if necessary, in the creation of national associations
- **AIM 5:** To promote research into Rett syndrome

Building the Network

- **AIM 1 of the statutes:** To make Rett syndrome better known to the public, professionals, carers [...]

1. Revitalise the network

Constant update of all the contacts in all countries to be able to communicate

2. Inform, advertise special events

News/Articles/Events on RSE website

Video of associations during last GA

Building the Network

- **AIM 1 of the statutes:** To make Rett syndrome better known to the public, professionals, carers [...]

Ms. Glòria Renom

Member of the Parliament of Catalonia

Mr. Thomas Bertrand

President of Rett Syndrome Europe

Dr. Josep Torrent

CEO of the foundation Dr. Robert

Dr. RoserValles

Advisor to the Office of the Ministry of Health

Ms. Monica Martinez

Chief of the Teknon Foundation

Dr. Jose MaPayà

Medical Director of the Medical Center Teknon

Mr. Jordi Serra

President of the Catalan Association of Rett Syndrome

Mr. D. Juan José García Fenoll

President of the Spanish Association of Rett Syndrome

On March 2, 2013 took place the 3rd Catalan Rett Syndrome Day: "Update on Research, Emotional Wellness, Disability and Guardianship at the Teknon Medical Center in Barcelona"



Building the Network



The Drug Information Association (DIA) 2013 Eurometing

by T.Bertand on 14 APRIL 2013 in CONFERENCES, EVENTS, NEWS

By Danijela Szili. The DIA (Drug Information Association) is global-wide working association which provides networking opportunities and information about innovation in science and pharmacy by organising events and training courses over the entire world. The biggest get-together on our continent this year was the Eurometing 2013 (March 6th RAI centre, Amsterdam) with more than 3,000 participants. [...]

[READ FULL STORY](#) • COMMENTS ARE CLOSED

3rd European Rett Syndrome Conference "Research Update and Preventive Management"

by T.Bertand on 27 MARCH 2013 in CONFERENCES, EVENTS

The 3rd European Rett Syndrome Conference Maastricht, "Research Update & Preventive Management" (ERSCM 2013), will take place on October 17th – 19th, 2013 at the MECC in Maastricht, The Netherlands. For more information on registration and program please visit: <http://www.ersmc.eu> ESCRM 2013 is an initiative of the GKC (Gouverneur Kremers Centrum), Stichting Terre (Dutch Rett Syndrome Foundation) and NRSV (Dutch Rett Syndrome Association) and [...]

[READ FULL STORY](#) • COMMENTS ARE CLOSED

VISIT US ON FACEBOOK



EUROPEAN EVENTS



ADVERT



Rett Syndrome is a complex neuro...
throughout their lives.
Sufferers are profoundly and multiply disabled and...
women, their families and carers.



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raduire Désactiver pour : finnois

Options

01/01/13 | Rettin oireyhtymä

Aure.fi sivustolle on avattu uusi keskustelualue. Keskustelualueelle pääsee klikkaamalla yläpalkissa olevaa Keskustelu -linkkiä. Foorumille pitää kirjautua, jotta pystyt kirjoittamaan ja vastaamaan viesteihin. Ulkoasu ja rakenne on vielä hieman kesken, muuten foorumi toimii normaalisti. Tervetuloa keskustelemaan ja jakamaan ajatuksia! Hyvää Uutta Vuotta!

1 2 3 4 5

European Rett Syndrome Congress
17 lokakuu

Katso kaikki tapahtumat

Viimeisimmät Kommentit

Sykeyn tapahtumat | AURE: Tapahtumat
Marjo: Keskustelu
päivi sippu: Keskustelu

Rett Syndrome Research Trust Blog

Life with Rett Syndrome – A Lost Friend

Boys With Rett Give Us Clues About
McCP2's Function

Rett Researchers Get Up Close and
Personal

FUNDING RESEARCH – FUNDING
RESULTS: RECORD \$4.2 MILLION
AWARDED

"Cleaning Up Science"

Rett Syndrome News

Rett Syndrome Awareness

Special Successes over Autism: Autism
Rett Syndrome Connection

New Rett Video Channel

Clinical trial for Rett syndrome launched
Rett Syndrome Video Channel: The
Premier Place for Rett Syndrome
Videos

Rett Syndrome Europe

The Drug Information Association (DIA)
2013 Eurometing

3rd European Rett Syndrome
Conference "Research Update and
Preventive Management"

Visit us on facebook!

Rett UK

Fundraising for Rett UK

Re UK Family Weekend

Rett UK's first ever blog, to celebrate
mel i Melanie Ekless, Chief Executive.

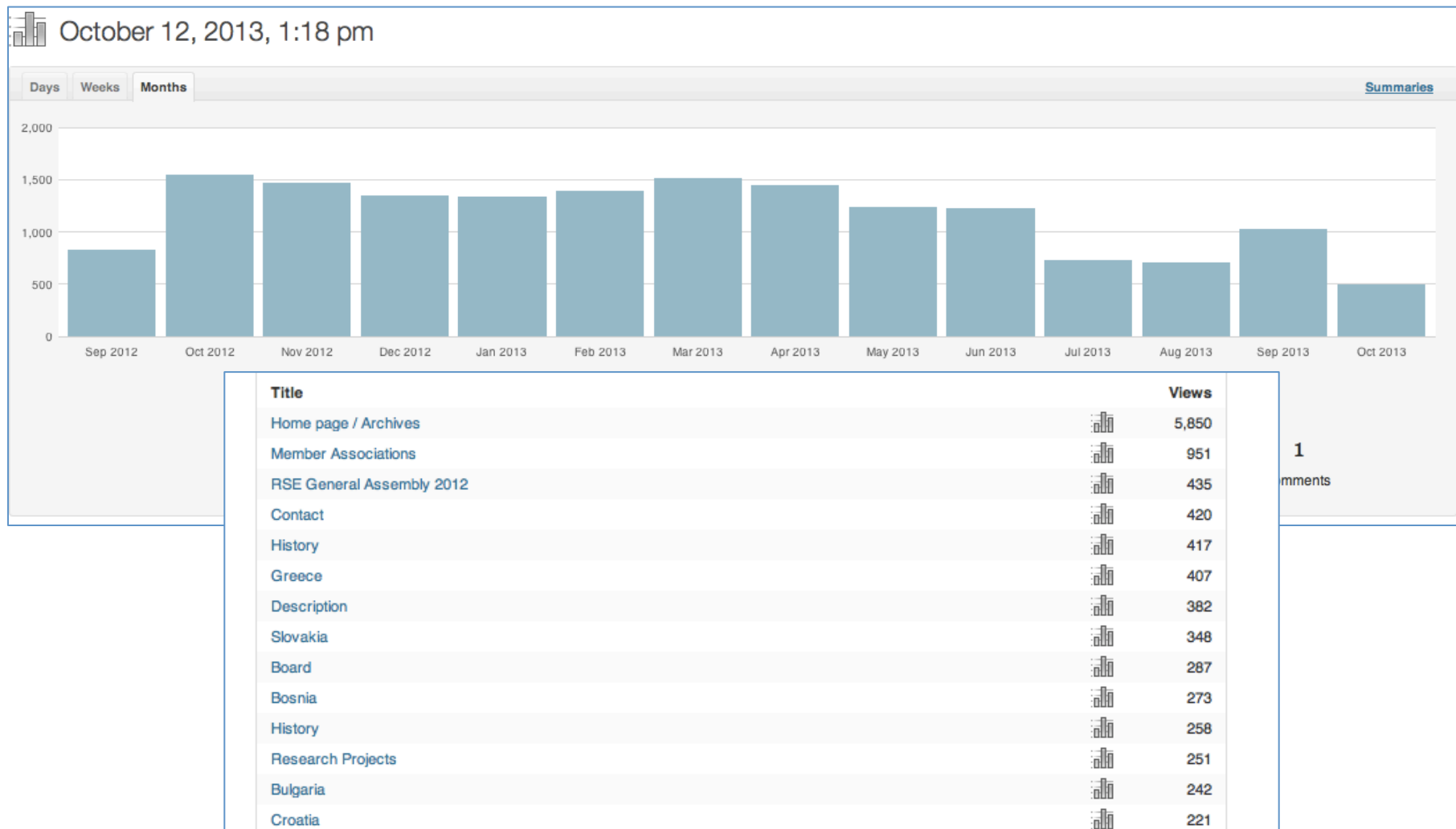
© 2013 AURE

<http://rettsyndrome.eu>

<http://aure.fi>



Website stats



Building the Network

- **AIM 2:** To improve the communication within the European Rett Community

→ **Discuss with other European parents and other Rare Diseases communities**

RareConnect (June 2012)

Facebook (March 2013)

Building the Network

des personnes, des lieux ou d'autres choses. Q

maintenir et indiquez que vous aimez en tant que Rett Syndrome Europe - Changer à Thomas Bertrand

Administration Notifications Modifier la Page Développer l'audience Aide Afficher

Maintenant Inscription sur Facebook

rse rett syndrome europe

303 j'aime 5 personnes en j'aime

Organisation à but non lucratif
Is a network of National Rett Associations which promotes research and aims to improve the quality of life of all individuals with Rett syndrome in Europe.

À propos Photos Mentions j'aime

Statut Photo / Vidéo Offre, événement +

Exprimez-vous

Rett Syndrome Europe a partagé un lien.
24 avril, à proximité de Budapest

<http://us2.campaign-archive.com/?u=c982ba6e2de2c3f943502a836d4f9e75dc2b0>

EURODIS shows: IDIRC conference advances research collaboration
us2.campaign-archive.com

Je n'aime plus Commenter Partager

Rett Syndrome Europe et 3 autres personnes aiment ça.

Écrire un commentaire...

Approuver sur Twitter pour publier

166 personnes ont vu cette publication 94 Stimuler la publication

Rett Syndrome Europe a partagé un lien.
13 avril

Did you know that the future drug therapies for Rett Syndrome as orphan drugs will have to go through Centralised authorisation procedure at the EMA (European Medicines Agency) in London in order to be marketed in EU?

Voir la traduction

What is an orphan drug?
Orphan drugs are medicinal products intended for diagnosis, prevention or treatment of life-threatening or very serious diseases

Je n'aime plus Commenter Partager

5 amis aiment Rett Syndrome Europe

Rett Syndrome Europe a partagé un lien.
17 avril

Be informed! Read about the most important events in the Rare disease field! <http://www.rareindiseaseblogs.net/>
Voir la traduction

Rare Disease Blogs: International opinion on Rare Diseases & Orphan Drugs
www.rareindiseaseblogs.net

RareDiseaseBlogs-Net is a blogging platform project and is a joint

Je n'aime plus Commenter Partager

Rett Syndrome Europe et 7 autres personnes aiment ça.

Écrire un commentaire...

Approuver sur Twitter pour publier

205 personnes ont vu cette publication 94 Stimuler la publication

Rett Syndrome Europe a partagé un lien.
13 avril

Watch this informative video about European Patients' Academy for Therapeutic Innovation, with Jan Geissler, EUPATI Director

Voir la traduction

About EUPATI, its objectives, benefits for patients - and its upcoming conference

1. What does EUPATI stand for? 2. What are EUPATI objectives? 3. How do patients and the public benefit

Faire Commenter Partager

BIENVENUE THOMASBERTRANDRETT | PROFIL | DÉCONNEXION

Communauté: **Le syndrome de Rett**

Langue: **Français**

Communautés de malades atteints de maladies rares

Community Home
Accueil / Le syndrome de Rett

Bienvenue –
Qu'est-ce que le syndrome de Rett?

Le syndrome de Rett est une maladie génétique qui affecte profondément la vie quotidienne.

martacampabadal | publié il y a 3 mois | Rédigé à l'origine en anglais
Clinical trial: Treatment of Rett Syndrome With Recombinant Human IGF-1
Investigators are recruiting children for a clinical trial using the medication recombinant human IGF-1 (a.k.a. mecasermin or INCRELEX) to see if it improves the health of children with Rett syndrome (RTT). While IGF-1 is approved by the Food & Drug Administration (FDA) for certain use in children, it is considered an investigational drug in this trial because it has not previously been used to treat Rett. Information from this study will help determine if IGF-1 effectively treats Rett but will... (Afficher plus)

Traduire en français:
Traduction instantanée de cette conversation (Google Translate)
Demande de la traduction humaine de qualité (ce message seulement)

Aucun signalement | Mettre hors ligne | Rendre privé | Supprimer | Modifier | **Réponse**

5 réponses à cette conversation — Voir toutes les réponses

pinivg | publié il y a 2 mois | Rédigé à l'origine en italien
Anche a Viareggio (Italia) è in corso una sperimentazione con IGF1 nella sindrome di Rett. Se desiderate ulteriori informazioni potete scrivirmi: g.pini@us12.lscana.it

Demande de la traduction humaine de qualité (ce message seulement)
Modifier | Mettre hors ligne

danieljaszili | publié il y a environ 1 mois | Rédigé à l'origine en anglais
It is all about assessing benefit/risk ratio. If FDA concludes after evaluating the results of this Clinical Trial that the benefits are higher than the risk (the adverse drug reactions), then the medicinal... (Afficher plus)

Demande de la traduction humaine de qualité (ce message seulement)
Modifier | Mettre hors ligne

stellaheckary | publié il y a environ 1 mois | Rédigé à l'origine en allemand
Leider konnte ich keine Verbindung mit dem link zu Dr. Kauffman bekommen. Ich nehme jedoch an, dass das Präparat - und der Versuch ist ja nur für die USA gültig - via Placebo versus IGF 1 Studie gemacht... (Afficher plus)

Demande de la traduction humaine de qualité (ce message seulement)
Modifier | Mettre hors ligne | **Réponse**

Les dernières
Conférence européenne Syndrome de Rett 2013
Stichting Terrie Foundation
bor d'après le...
publié il y a 1 an
Aucun commentaire
L'ERSO du GKC (Fondation du Syndrome de Rett)

Partenaires et
RareConnect est un partenariat de

Rett Syndrome Europe
Asoc. Española de Rett Syndrome de Rett
Asoc. Nacional Amigos Rett
Landesforeniner Rett Syndrom
Österreichische Rett Syndrom Gesells
Asoc. Française Syndrome de Rett

Raptor Pharmaceutical Licenses IP for Rett Syndrome from French Research Institutes
sujet publié il y a environ 20 heures
Voir la transcription

ULZIBAT - Methode
sujet publié il y a 4 jours
Voir la transcription

Statins improve symptoms of Rett syndrome in mice
réponse publié il y a 4 jours
Voir la transcription

L'huile de poisson comme "traitement" pour le syndrome de Rett
document publié il y a 22 jours
Voir la transcription

Phase 2 Study of EPI-743 for Treatment of Rett Syndrome in Italy
sujet publié il y a environ 1 mois
Voir la transcription

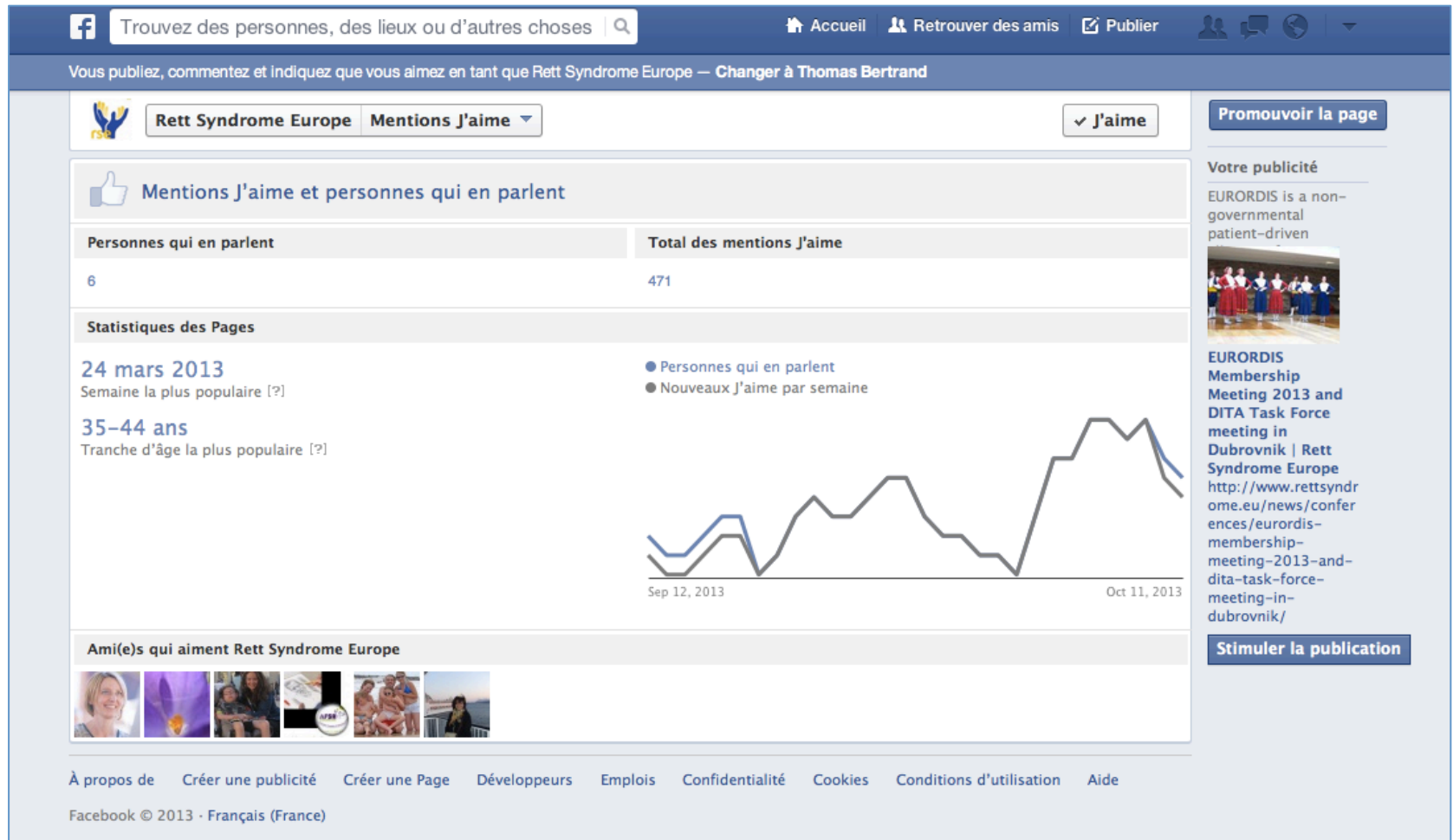
Clinical trial: Treatment of Rett Syndrome With Recombinant Human IGF-1
réponse publié il y a environ 1 mois
Voir la transcription

Life with Rett Syndrome: It is so much more than the facts, the stats and the science
sujet publié il y a environ 1 mois
Voir la transcription

Life with Rett Syndrome
réponse publié il y a environ 1 mois
Voir la transcription

<https://www.rareconnect.org/en/community/rett-syndrome>

Facebook statistics



RareConnect statistics (since June 2012)

RSE Moderators: Danijela Szili, Stella Peckary, Thomas Bertrand

- Since its creation the Rett Syndrome community has:
 - **190** members
 - **22** patient groups featured
 - **16** articles
 - **12** testimonials
 - **50** forum topics
 - **6** Documents
- **Who has visited the Rett Syndrome Community since its creation?**
- **7057** unique visitors
- **Where are visitors coming from?**
 - Top 10 countries with most visitors:
 - 1. United States 1,726
 - 2. Italy 683
 - 3. France 572
 - 4. United Kingdom 487
 - 5. Germany 477
 - 6. Spain 432
 - 7. Canada 224
 - 8. Australia 204
 - 9. Mexico 176
 - 10. Netherlands 119
- **How are people finding the community on search engines?**
 - Top 10 keywords (the words people use on a search engine to find the community):
 - mi princesa rett
 - rareconnect
 - sindrome di rett
 - rett syndrom
 - ipad apps for rett syndrome
 - syndrome de rett
 - ulzibat
 - sindrome de rett
 - rett syndrome apps
 - 3rd european rett syndrome congress
- **How did visitors find the community?**
 - [1.google](#) 3,381
 - [2.\(direct\)](#) 1,370
 - [3.facebook.com](#) 901
 - [4.m.facebook.com](#) 410
 - [5.rettysyndrome.eu](#) 269
 - [6.eurordis.org](#) 226
 - [7.bing](#) 91
 - [8.twitter.com](#) 89

External Influence and Advocacy

- **AIM 3:** To promote as a representative European organisation, the interests of people with RTT and families
 - *Having RSE officially in the network of European institutions*
- 1. **RSE is a member of EURORDIS:** Allows RSE to vote at the GA of EURORDIS During the EMM (EURORDIS Membership Meetings), *Martine and Danijela in Dubrovnik (June 2013)*
 - *EURORDIS fellowship was granted for Hungary, Croatia and neighbouring countries to attend this EMM*
 - *RSE was granted funds in EURORDIS “Support Rare Disease Federations” 2013 call for Eastern European countries to be able to attend today’s RSE General Assembly*

External Influence and Advocacy

Rare Diseases: an EU Public Health Priority

Improving access to quality care

Improving access to Orphan Drugs

Deal with ethical issues



Promote Research and link patients to professionals across country borders

External Influence and Advocacy

2. EURORDIS Training resources:

- Summer School June on « patient advocates in clinical trials and drug development » June 2013, Barcelona, *Liana Murtazina (Russia)*
- Online learning tools
 - clinical trial and orphan medicinal products

3. EURORDIS Task Force:

- DITA Drug Information and Transparency Access Task Force June 2013, Dubrovnik, *Danijela*

4. EUCERD: European Union Committee of Experts in Rare Diseases – *Gérard Nguyen*

External Influence and Advocacy

- 5. **EMA** European Medicine Agency (London, UK): RSE fulfils the criteria to be involved (letter of 14/11/11)
 - Training programs for patients and patients advocates: Pharmacovigilance Workshop, *Danijela*
 - PCWP: Patient and Consumer Working Group, *Danijela (Training Sessions)*
 - *RSE applied to the Call for expression of interest to eligible organisations to become a member of the PCWP – not granted 2013-2016*
 - PDCO: Paediatric Committee
 - *New call for PDCO members from Academia / Health Care / Patient Representatives – deadline 8 November*

External Influence and Advocacy



Danijela Szili
(President of the Hungarian
Rett Syndrome Foundation)

DITA member (Drug Information, Transparency and Access)



External Influence and Advocacy

6. DIA: Drug Information Association: Annual Eurometing March 2013, Amsterdam,
Danijela

- *RSE applied for a DIA Philanthropy Grant Application for the translation of a book to be published by AFSR – Not granted*

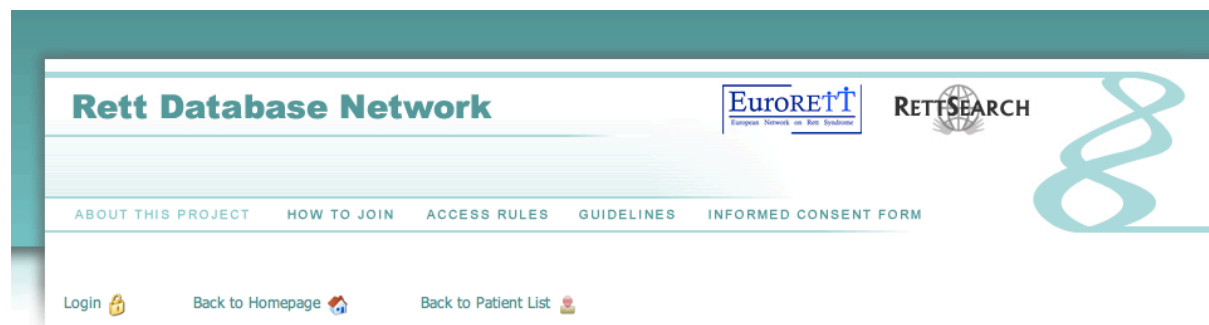
7. EUPATI: European Patients Academy on Therapeutic Innovation: Workshop April 2013, Roma, *Olga Timutsa (Russia)*

Our Network

- **AIM 4:** To Expand RSE to all European Countries and to assist, if necessary, in the creation of national associations
 1. Russia
 2. Greece
 3. Romania
 4. Iceland
 5. Macedonia (contacts)
 6. Bulgaria (contacts)

→ 39 contacts

AIM 5: To promote research into Rett syndrome



Number of patients in archive: 1904

Australia 1	France 252	Italy 601
Czech Republic 0	United Kingdom 255	Poland 0
Germany 0	Croatia 29	Portugal 0
Denmark 64	Hungary 58	Romania 15
Spain 387	Israel 93	Sweden 0
Finland 0	India 3	USA 96
	Serbia 50	Russia 0

<http://www.rett databasenetwork.org>

Oliviero Dell'Oro (Italy)
Jordi Serra (Catalonia)

«The aim of this project is to connect the already existing databases and to create a unified repository [...] The data will be accessible to the participants and to the scientific community according to rules that assure transparency and equity [...] This international effort will be of great value in order to perform genotype-phenotype correlations, to study modifier genes, and to select subgroups of patients for clinical trials.»

Challenges and questions

- We now have the Network (we can still improve)
- We now have tools to advertise (Website)
- We have tools to communicate (Rareconnect/ facebook)
 - need for others? (twitter) – time consuming
- What's next: how can we use the network with the tools?
- New RSE Roles? Fundraising? For What? How?
 - (Role in advocating for research and/or care issues?)