EURORDIS
RARE DISEASES EUROPE

Therapeutic Action Group

a bridge between the rare disease community and the EMA Committees

EMM 2021

Wednesday 12 May

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Who's the TAG? i.e. Your representatives on EMA Committees

Julian Isla Gomez
European Dravet Syndrome Federation
COMP since 2014
(*observer then member since 2018*)

Pauline Evers
EGAN & Dutch Cancer Association
COMP since 2006
(*member*)

Loris Brunetta
Italian Foundation for the cure of Thalassemia & TIF + ePAG EuroBloodNet
COMP since 2018
(*member*)

Maria Cavaller
EURORDIS
Protocol Assistance – collaboration with EMA public engagement team

Dimitrios Athanasiou
Duchenne UPPMD
PDCO since 2017
(*member nominated by EURORDIS, supported by WDO and EPF*)

Tomasz Grybek
Fundacja Bohatera Borysa + ePAG ITHACA
PDCO since 2020
(*alternate*)

Cathalijne Van Doorne
EFNA (*PCWP member*) + ePAG ERN-RND
PRAC since 2019
(*member*)

Virginie Hivert
EURORDIS
PRAC since 2019
(*alternate*)
COMP since 2014
(*Expert*)

François Houÿez
EURORDIS
PCWP

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Poll 1

Please tell us how many Committees there are at the EMA (European Medicines Agency)?

- 5
- 7
- 9

Poll 2

Amongst these Committees, please select the ones which have patients as full members in their composition (multiple answers possible)?

- COMP for Orphan Medicinal Products
- PDCO for Paediatric Medicines
- CAT for Advanced Therapies
- CHMP for Benefit-Risk Assessment and Market Authorisations
- CVMP for Veterinary Medicines
- HMCP for Herbal Medicines
- PRAC for Pharmacovigilance & Risk Assessment

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

- Call for expression of interest issued by the European Commission
- EURORDIS proposing and/or endorsing applicants
- Mandates are for a duration of 3 years
- Not all the patients on the EMA Committee are included in the TAG
- As much as possible, we try to include all patient representatives from the rare disease community to ensure cross-talks and common approach

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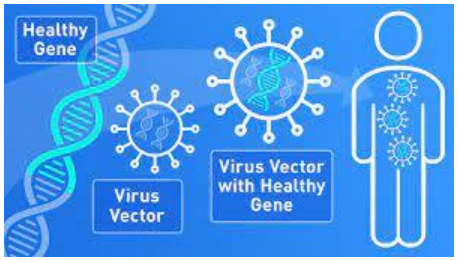
If you would like to apply to the next calls coming up around June/July i.e. CAT and PRAC

CAT for Advanced therapies



PRAC for Pharmacovigilance & Risk Assessment

Contacts – Maria Cavaller (maria.cavaller@eurordis.org) and/or myself (virginie.hivert@eurordis.org)

COMP – for Orphan Medicinal Products

Established by the *Reg (EC) No 141/2000 of the European Parliament and of the Council on Orphan Medicinal Products of 16 December 1999*

3 Patient representatives as Members

Orphan designation, Maintenance of the orphan status at the time of Marketing Authorization, Protocol Assistance

Definition of medical conditions, prevalence below 5 in 10,000 in the EU, medical plausibility of the proposed product, satisfactory methods already available, significant benefit (including major contribution to patient care)

Ad-hoc patient experts are invited for some procedures

Currently: emphasis on the Impact Assessment of the Orphan & Pediatric Regulations in the context of the pharmaceutical review

PDCO – for Paediatric Medicines

Established by the *The Paediatric Regulation which came into force in 2007*

3 Patient representatives as Members and 3 as Alternates

Paediatric Investigation Plans, waivers, deferrals in relation with the development of medicines for children (eg design of trials), scientific advice.

Currently: emphasis on the Impact Assessment of the Orphan & Paediatric Regulations in the context of the pharmaceutical review

PRAC – for Pharmacovigilance & Risk assessment

Established by the *The Pharmacovigilance Regulation which came into force in 2012*

1 Patient representative as Member and 1 as Alternate

Signals, safety issues, risk management plans, post-authorisation safety studies, referrals.

Currently: COVID-19 vaccines !!

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Beyond Committees - TAG in short

Meeting every month to go over the main topics discussed in each Committees and align amongst ourselves and with the needs of the RD community

Work on transversal topics with EURORDIS colleagues, such as on the Impact Assessment of the Orphan & Paediatric Regulations in the context of the pharmaceutical review

Watch for opportunities for patient engagement at the EMA such as Protocol Assistance, SAG (Scientific Advisory Group), new opportunity at CHMP level for early interaction with patients

TAG acts as a bridge between the rare disease community and the EMA Committees therefore…

It is important for us to **get insights on the expectations from the patients** vis a vis of a product, even before it comes to EMA, the assessment of the **unmet medical needs** in the various disease areas, etc

We need you! Please get in touch!

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