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Motivation Letter and Program Outline

Application for the ESID registry chairperson 2022-2026

Graz, June 17, 2022

Dear colleagues,

Herewith I am applying for the position of the chairperson of the ESID registry working party, and I am declaring my willingness to participate and contribute actively in the development and future of our society.

I have been participating in the ESID registry as national coordinator in Austria for the majority of its soon 20-year anniversary existence and *active within the registry steering committee* / working group for over 8 years. Being a pediatric hematologist-oncologist with strong interest in immunodeficiencies and clinical research, I consider it of utmost importance to have an international network of centers and experts for primary immunodeficiencies as well as reliable datasets of patients with rare diseases at hand that allow joint research, development, and improvement of patient care.

In the recent history of the ESID registry, I conceptualized and put into effect two "*buttons*" into level 1 documentation, *i.e.*, one for immune modifying drugs and one for malignancies –a balancing act between simple (1-3-click) collections of basic data or too detailed or complicated queries– to enable designing more specific research studies based on this, in my opinion, important preselection. Furthermore, together with *G. Kindle* and the then current and preceding steering committees and many contributors, I summarized the reasons, benefits, and results of the implementation of clinical *diagnostic working definitions for IEI*, which became the most cited paper of *JACI-in practice* within a short time. *"Using and improving the ESID registry"* has been my clinical research dogma within the last decade.

Entering data into an online registry should never be felt as one-way track. On the contrary, it should pay off immediately, and multiple times over, for the participants all over Europe and collaborators globally. During the next four years, if I am elected, I would therefore dedicate my work focus on:

1) Improving the direct benefit from completing datasets for all participating centers;
2) Facilitating the possibilities to address own specific research questions fast – for anyone with a clear research idea and study concept;
3) Enhancing the networking possibilities to connect physicians with experts in very rare IEI based on registry entries.

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Although the registry is going through a transition with some of many issues still to be resolved, I am positive to take over, if elected, the largest international patients' registry in IEI with an extremely promising potential for all of the focus areas I mentioned. I think that besides consolidating and actualizing the legal framework and the technical platform, another prerequisite is the strengthening of the IT-resources to accelerate return speed and improve the still exploitable many potentials of the registry, which is so central to our society and both internal and external stakeholders.

Some of the specific aims I would strive to achieve are:

1) *Automated delivery of center reports* with the most relevant epidemiological facts of your center, recent changes, and comparisons with neighboring countries and the entire registry, useful for your own data and patient management;
2) Generation of an automated *personal network function on specific IEI*, connecting experts in a specific rare IEI on voluntary (opt-in) basis with those who newly added patients with the same IEI to facilitate joint research and improvement of patient care;
3) Restriction of the level 1 dataset to what it was redesigned for ("*keep it simple*"), namely solely a basis for more specific level 2 and level 3 studies, the latter two of which should be facilitated through strengthened office and IT-resources and a *modular and versatile technical structure*;
4) Provide a platform for treatment optimization studies while maintaining the status of a non-interventional, observational registry, *e.g.*, by offering *disease-specific patient subregistries* for therapy guidance studies (similar to HLH);
5) Launch an *update of the ESID clinical working definitions for genetically undefined IEI* as large joint effort with IEI experts;
6) Integration of critical data from *newborn screening* for severe combined immunodeficiencies;
7) Integration of HPO (*human phenotype ontology*) terms into certain ESID registry functions where reasonable;
8) Close collaboration with the *genetics* and *inborn errors working parties* as well as with the *EBMT* and *Newborn Screening programs* to increase synergies in research efforts in IEI and mutually improve output;
9) Maintenance of full compliance with EU-GDPR requirements of the registry infrastructure, of its interaction with participating centers and patients, with as minimal bothering of end users and patients as possible;
10) *Reward high-performers* (centers with highest number of entries per inhabitants, highest proportion of complete follow-ups, highest frequency of reconsented patients, etc…) with, *e.g.* a travel grant or waiver for registration for the next biennial ESID meeting (board approval pending ;)).

These aims will need time and not only my personal effort to be successful! But for the start, I need your support to put any of these steps further into action. With this I would like to thank you for your interest and contribution to the ESID registry so far and invite everyone to communicate her:his additional ideas for the use and improvement of the ESID registry to me!

Thank you for considering my application,

Markus Seidel