



Mind the Gap!

Multi-stakeholder dialogue for priority setting in health research Pilot project: Tuberous Sclerosis Complex King Baudouin Foundation Belgium

What is it about?

The Belgian King Baudouin Foundation, be-TSC and the University Hospital of Brussels will bring together the voice of people with tuberous sclerosis complex (TSC) and their relatives, with professionals providing health and paramedical care, researchers and representatives from potential research sponsors to identify and rank treatment uncertainties and prioritise future research in TSC.

Tuberous sclerosis complex

Tuberous sclerosis complex (TSC) is a genetic disorder affecting multiple organs, including skin, brain, heart, kidneys, eyes and lungs. Although there is significant variability in the number and severity of clinical features, the most common pathological manifestation is the formation of benign tumours in these organs, seizures and TSC-associated neuropsychiatric disorders, which can include autism spectrum disorder, cognitive disability, anxiety and depression TSC affects approximately 1 in 6,000 to 10,000 individuals and is caused by mutations in the TSC1 or the TSC2 gene. Although TSC can be inherited as a dominant disorder, two thirds of patients have new mutations (de novo mutations) and thus did not inherit the disease from one of their parents. Appropriate management and coordination of specialist care, both medical and non-medical, is crucial across the lifespan of children or adults with TSC to reduce both morbidity and mortality.

Why research priority setting in TSC?

Considerable progress has been made in TSC research, but do the current research agendas sufficiently match the needs of the patients and their relatives, or of their doctors and other caregivers? The specific knowledge and expertise of some of these groups is often undervalued and underused when it comes to prioritisation of research topics.

Multi-stakeholder dialogue

A multi-stakeholder dialogue is a way of consulting parties who are seldom being heard. It is a multi-actor and iterative process in which patients and parents work together with researchers, caregivers, and research sponsors to identify treatments uncertainties and prioritize research topics.

In the first round, each group will separately identify uncertainties relating to the prevention, diagnosis, treatment and care of TSC. Methods of gathering information will be adapted according to the nature of each group, ensuring transparency, inclusiveness and representativeness. In addition, existing sources of information concerning such uncertainties and research priorities in TSC will be used.



In the second round, participating groups will prioritise these uncertainties and research topics through dialogue in a face-to-face consensus meeting.

Participating groups in the TSC research priority setting exercise, include:

1. Belgian patients with TSC, parents of patients, be-TSC
2. National and international TSC patients' organisations
3. Clinical, paramedical and social care experts in TSC
4. TSC researchers and researchers in related fields
5. Research funders and sponsors

Expected outcomes

This exercise will result into a ranked list of treatment uncertainties translated into priority research questions, inspired by Belgian TSC patients, parents, caregivers and researchers, strengthened and endorsed by their international peers and thus embedded in an international context.

The outcomes will be described in a report by the King Baudouin Foundation and in a scientific paper published in a peer reviewed international journal.

Who-is-who

King Baudouin Foundation - Working together for a better society

The King Baudouin Foundation is an independent, pluralistic foundation working at the local, regional, federal, European and international levels. Because our mission is to *change society for the better*, we invest in inspiring projects and individuals. In 2015, the King Baudouin Foundation and the Funds it administers provided €34.8 million to support 264 individuals and 1,813 organizations. Projects were funded in the areas of poverty, health, development, civic engagement and heritage, among others.

For KBF, **Annemie T'Seyen** and **Bénédicte Gombault** are supervising the project 'Research priority setting for TSC via multi-stakeholder dialogue'.

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be-TSC

be-TSC wants to be there for anyone in Belgium who is concerned by tuberous sclerosis complex. be-TSC informs, supports and connects through a web page, an active facebook group and direct contact. The organisation raises awareness of TSC in society because receiving recognition for what marks one's life, helps in making people more resilient. Furthermore, societal recognition of TSC is a first step towards better understanding and care. The president of be-TSC and the be-TSC face for this priority setting project is **Eva Schoeters**.

www.betsc.be



UZ Brussel

With its 700 beds, each year UZ Brussel admits more than 25,000 patients and treats 400,000 outpatients, both national and international. The hospital's clinical excellence, scientific commitment and human-centric tuition ensure the highest degree of competence in many fields. The hospital is closely associated with the Vrije Universiteit Brussel (Free University Brussels).

Prof. **Anna Jansen** from the Pediatric Neurology Unit organises a multidisciplinary TSC consultation at UZ Brussels, conducts TSC research and is also the initiator of the TSC research priority setting project.

