Regions4PerMed

# Interregional Coordination for a fast and deep uptake of Personalised Health - Regions4PerMed

## *Best Practices Booklet*

**Key Area 3: Personalising Health Industry**

HORIZON 2020

This project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 825812

**PROJECT INFORMATION**

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| GRANT AGREEMENT NUMBER | 825812 |
| PROJECT FULL TITLE | Interregional coordination for a fast and deep uptake of personalised health |
| PROJECT ACRONYM | Regions4PerMed |
| FUNDING SCHEME | CSA |
| START OF THE PROJECT | 01/11/2018 |
| DURATION | 54 Months |
| CALL IDENTIFIER | H2020-SC1-2018-Single-Stage-RTD |
| PROJECT WEBSITE | http://www.regions4permed.eu/ |

# Best Practices booklet

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## 1. Codex4SMEs – Companion Diagnostics expedited for SME

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| Project \| Initiative Title \| Logo(s) | | Codex4SMEs     |
| Organisation name | | BioRegioSTERN |
| Country | | Germany |
| Region | | Baden-Wuerttemberg |
| Contact person | | Dr. Margot Jehle |
| Contact email | | jehle@bioregio-stern.de |
| Website: | | www.nweurope.eu/codex4smes |
| Keywords: | | Companion Diagnostics, Diagnostics, Personalized Medicine, SMEs, European network, support services |
| Duration: | | December 2023 |
| Area of application | | Diagnostics – Covid-19 diagnostics included |
| DESCRIPTION | Main challenges tackled (max 200 words) | NWE territories are lagging behind in the world-wide implementation of PM and CDx (enabling cost-savings in public healthcare) due to the lack of an appropriate support infrastructure for SMEs in this field. SMEs within the (Companion) diagnostics field are among others facing problems with the access to biosamples; they are lacking experience and knowledge within biomarker validation process, access to different stakeholders, access to funding and investors, access to and cooperations with big pharma companies, regulatory and reimbursement issues, entering further European markets. |
| | Objectives (max 200 words) | The original project aimed to establish a transnational network with 9 project partners from 7 regions for SMEs able to expedite time-consuming & costly CDx development via linking of stakeholders at all levels and increasing the TRLs of SMEs´ products up to 7-8 by providing a tailored support scheme for SMEs. Within the capitalisation phase Codex4SMEs enlarges its established network by the involvement of 3 new partners. The original support scheme for SMEs will be modified into a new Fast-Track Programme to further expedite the time-to-market of novel diagnostic solutions and to be applied for a broadened sector of diagnostics in general with a special focus on COVID-19 diagnostics. Thus, supported SMEs gain significance in international markets and increase their global competitiveness. |

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| | Main concept and methodologies involved (max 200 words) | The original project Codex4SMEs offered a tailored support scheme for SMEs encompassing a sample access & a knowledge transfer service, a Biomarker validation, access to networks/ecosystems from seven countries, transnational roadshows with venture capitalists and large pharma companies, expert advice regarding business model, business growth and upscaling. The Codex4SMEs capitalisation phase will offer within its new Fast-Track Programme further services such as seminars on biomarker topics, tailored consultancy on specific biomarker topics on SMEs´ needs, modular biomarker validation services, partner search to identify the best academic collaborator with the right scientific & medical expertise, translational assessment as service to assess the translational pathway and feasibility of projects, full regulatory assessment/scientific advice, access to pharma and big medtech companies within a so called Meet&Match.Dx service, support services for business growth of SMEs (e.g. for market analysis or reimbursement issues).<br><br>Support services will be tailored according to the SME's current stage of development. |
| | Impact (health, scientific, industrial, socio-economic or others enabled by the project/initiative (max 200 words) | 297 SMEs have been involved within the Codex4SMEs project so far. The key outcomes for the SMEs have been:<br><br>further development of products, international contacts / access to biobanks, experiences in pitching, R&D collaborations. As a result, SMEs could increase their turnover, the number of jobs and the TRL level of their products.<br><br>Therefore, the project has been contributing to an increased implementation of personalised medicine and healthcare and will furthermore support this implementation within its capitalization phase by offering the new Fast-Track Programme. |
| | Funding and Investment (please specify the source: public, private, Structural or other types of funds) | ERDF funding of in total 2.5 Million Euro |
| | Key stakeholders involved | BioRegio STERN (DE), BOM (NL), Innovation Quarter (NL), WestBIC (IE), Medicen (FR), IBBL (LU), EATRIS (NL), EIT Health Germany (DE), Flanders.bio (BE) |

## 2. EKFZ – Else Kröner Fresenius Center for Digital Health

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| Project \| Initiative Title \| Logo(s) | Else Kröner Fresenius Center for Digital Health |
| Organisation name | Technische Universität Dresden & University Hospital Carl Gustav Carus |
| Country | Germany |
| Region | Saxony, Dresden |
| Contact person | Sabine Marschollek, Head of Administration & Project Coordination |
| Contact email | ekfz@tu-dresden.de |
| Website: | https://digitalhealth.tu-dresden.de/ |
| Twitter: | @EKFZdigital |
| LinkedIn: | @Else Kröner Center for Digital Health |
| Keywords: | eHealth, digital health, Living Lab, Hospital 4.0, Robotics, Implants and Sensors, Connected Care, Artificial Intelligence, Digitization, Innovation, Interdisciplinarity |
| Duration: | 2019-2029 |
| Area of application | |
| DESCRIPTION – Main challenges tackled (max 200 words) | Wireless communication, new sensors, robotics, machine learning and artificial intelligence offer substantial potential for better patient care, personalized medicine and a smarter medical workplace. In contrast to established and well-funded molecular research structures, the interface between technology and medicine is scientifically and structurally under-developed. Thus, the digital revolution fails to fully deliver its benefits to clinical science and patients. To enhance innovation in the field of medical digital health, we need specialists who understand and interact constructively and creatively with each other. The EKFZ for Digital Health brings digital innovation to the patient by creating a new interdisciplinarity between medicine and engineering.<br><br>Using the knowledge of various disciplines will revolutionize medical care and offers more precise and effective treatments to patients, as medical treatment is tailored to account for individual differences and singular diagnosis. By tailoring the right medical treatment for a specific patient, therapies could become more successful with less side effects responding better to patients needs. An important goal is the establishment of an academic nucleus and the setting up of a supporting infrastructure for implementation and transfer. |

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| | Objectives (max 200 words) | The EKFZ for Digital Health aims at enhancing a generation of physicians with comprehensive technical knowhow and skills and vice versa engineers with a thorough understanding of medical and patient needs. When working with us, physicians and engineers will gain a holistic understanding of the future patient care. We enable them to learn from and work with each other to overcome the barriers of disciplines for effective implementation of medical innovation. Patient-focus is the core of center mission and is taken into account and considered in all EKFZ activities.<br><br>The EKFZ for Digital Health is an interface between technology and medicine that intends to set scientifically and structurally standards. The center supports future-oriented interdisciplinary innovation research projects. These intend to shorten the time-span from the initial idea to the prototype and thus generate a faster benefit for the patient. As the digital revolution offers great chances for novel therapies and diagnostic technologies, the benefit for patients is getting closer to personalized therapy (new sensors, personalized implants, 3D print). |
| | Main concept and methodologies involved (max 200 words) | The EKFZ for Digital Health is a joint cross-faculty initiative at the Technische Universität Dresden, the University Hospital Carl Gustav Carus Dresden along with several Fraunhofer, Helmholtz and Leibniz institutes on the Dresden campus. Aiming for the benefit of the patient, the School of Medicine and the high-tech specialists on campus bundle their expertise in an initiative driven by medical need and with direct access to medical infrastructure. Whereas conventionally these different disciplines work and research independently, the EKFZ for Digital Health brings them together in training, undergraduate and postgraduate research. The center creates a unique and physically tangible interdisciplinary environment. This interdisciplinarity spans from joint teaching over joint professorships to joint projects.<br><br>The EKFZ provides scientific infrastructure, advice in terms of data integration and security, implementation and regulatory affairs. Scientifically the center funds innovation projects that focus on digital interface agendas, placing a strong focus on open and competitive interdisciplinary innovation packages to reduce the time from idea to prototype and patient. In order to explore digital innovation early on in patient context, we utilize infrastructure in a direct patient care and research context by implementing a 'Living Lab' enclosed in the University Hospital Dresden. |
| | Impact (health, scientific, industrial, socio-economic or others enabled by the project/initiative (max 200 words) | A structured interdisciplinary unit directly on the Medical campus, facilitated patient access and a joint training environment for physicians, software engineers and medical technology engineers with a broad and open technological profile marks the highly innovative structure of the EKFZ. Because the center is based directly on the Medical campus, we can implement innovations faster at the patient interface than other initiatives. The EKFZ for Digital Health also serves as an academic and economic innovation nucleus for further digital health activities and investments from outside. Product development, clinical trials and specific implementation studies will attract additional funding from public research funding agencies, industry and health care providers. A strong expertise for regulatory, ethical and implementation issues provides efficient support for novel ideas and products. |

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| | Funding and Investment (please specify the source: public, private, Structural or other types of funds) | Initial funding comes from the private Else Kröner-Fresenius Foundation, Bad Homburg, for which the TU Dresden successfully applied. In a tough international review process, the center was selected for funding from initially 27 applications from all universities with well-known medical schools in Germany in 2019. The funding is supplemented by additional support from the Free State of Saxony. |
| | Key stakeholders involved | Technische Universität Dresden, Faculties of Medicine, Computer Sciences and Electrical Engineering, University Hospital Carl Gustav Carus Dresden |

## 3. EMSCO – European Myelodysplastic Syndromes Cooperative Group

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| Project \| Initiative Title \| Logo(s) | | EMSCO – European Myelodysplastic Syndromes Cooperative Group<br>  |
| Organisation name | | EMSCO – European Myelodysplastic Syndromes Cooperative Group |
| Country | | Germany, France, Italy, Spain, Netherlands, Czech Republic, Switzerland, Austria, UK |
| Region | | Europe |
| Contact person | | Prof. Uwe Platzbecker, Silke Gloaguen |
| Contact email | | silke.gloaguen@medizin.uni-leipzig.de |
| Website: | | https://www.emsco.eu/ |
| Keywords: | | Myelodysplastic Syndromes (MDS), collaboration, clinical trials, synergies |
| Duration: | | Since 2013, undetermined duration / ongoing |
| Area of application | | Clinical trials, scientific projects and meetings/education in MDS |
| DESCRIPTION | Main challenges tackled (max 200 words) | MDS is a rare disease with heterogeneous subgroups – targeted clinical trials are difficult to recruit. Cross-border trials are necessary to recruit patients but harbour challenges:<br>- Different regulatory environments between countries<br>- Language barriers between counties and study staff<br>- Different budget requirements<br>- Etc… |
| | Objectives (max 200 words) | EMSCO has the aim to offer an internationally oriented team to support scientific MDS team throughout Europe in order to provide a platform to conduct investigator-initiated trials (IITs) in an academic setting across countries with indication-specific expertise and knowledge of the various regulatory environments. |
| | Main concept and methodologies involved (max 200 words) | Project managers with MDS expertise and a collaboration platform for European MDS experts with an interest in clinical research and collaboration. |
| | Impact (health, scientific, industrial, socio-economic or others enabled by the project/initiative (max 200 words) | To date, 5 clinical trials in MDS have been completed in the context of the EMSCO platform, 3 are ongoing and 4 trials are in the pipeline. The platform has reached visibility – various interested stakeholders (both academic and |

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| | | industry based) have started to approach EMSCO for collaboration and value the synergies the platform has created. |
| | Funding and Investment (please specify the source: public, private, Structural or other types of funds) | EMSCO initially received funding from the European Leukaemia net in 2013. Since then, the platform has sustained itself by acquisition of study funding and support/sponsoring for meetings and educational activities mainly through the pharmaceutical industry. |
| | Key stakeholders involved | MDS experts and national MDS study groups from the countries named above. |

## 4. Medical Forge Leipzig – Bridging the Gap between Start-ups and Clinics

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| Project \| Initiative Title \| Logo(s) | | MEDICAL FORGE Leipzig  |
| Organisation name | | Biosaxony Management GmbH |
| Country | | Germany |
| Region | | Saxony |
| Contact person | | André Hofmann |
| Contact email | | gf@biosaxony.com |
| Website: | | www.medicalforge.de |
| Keywords: | | Accelerator, smart medical devices, regulatory, reimbursement |
| Duration: | | 2022 – 2025; planned to be a continuous program afterwards |
| Area of application | | |
| DESCRIPTION | Main challenges tackled (max 200 words) | The program is dedicated to local as well as international companies that need support to enter the German healthcare market. The MEDICAL FORGE Leipzig (MFL) provides basic lab and office infrastructure for a first foot print in the market as well as an extensive network of all stakeholders involved in bringing a new medical product to the market. Through direct cooperation with local clinics and private hospital groups MFL can assist with product validation as well as with implementation with health care providers. As large MedTech companies are involved in the program, it offers an easy access to decision makers for initiating cooperations, venture deals or distribution agreements. Health insurances connected with MFL will guide and assist |
| | Objectives (max 200 words) | The main objective is to improve the quality of health care in the region through implementing innovative technological solutions. Other objectives are the support of innovative companies that struggle with the "last mile" of product development – without putting an additional burden on the companies by taking shares or high service fees.<br><br>Our KPI are:<br><br>- Initiated and funded collaboration with R&D institutes |

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| | | - Initiated collaboration with local companies<br>- Initiated collaboration with clinics (e.g. validation studies)<br>- successful market access of participants |
| | Main concept and methodologies involved (max 200 words) | Based on a competitive selection process, 8 participants per year will be chosen to join the program. Applicants have to prove the medical need that is being served and a basic proof of concept for the products and services developed upfront. At the beginning of the program, we will evaluate the status of each project. Based on that, milestones for the collaboration within the MEDICAL FORGE will be worked out together. A curriculum for workshops and seminars on specific topics will be set up by MEDICAL FORGE (e.g. Pathways to the German Healthcare System). The MEDICAL FORGE will provide support to install QM-systems, address regulatory affairs and health economics. If this is not sufficient for specific requests and needs we bring in external experts that act as mentors or coaches for the teams. |
| | Impact (health, scientific, industrial, socio-economic or others enabled by the project/initiative (max 200 words) | The main impact is the acceleration of product development reducing the time to market. At the same time MEDICAL FORGE assists to improve quality of healthcare by making innovative products and services available. |
| | Funding and Investment (please specify the source: public, private, Structural or other types of funds) | The program is funded by the federal ministry of economic affairs and climate action. Source of funding is a special support for regions suffering from structural changes due to the phase out of lignite mining.<br><br>Besides that, several partners support the MEDICAL FORGE with financial or in-kind contributions. |
| | Key stakeholders involved | University Clinic Leipzig<br><br>St. Georg Hospital Leipzig<br><br>B.Braun Avitum Saxonia GmbH<br><br>LGH Leipziger Gewerbehof GmbH & Co. KG<br><br>HP Deutschland GmbH<br><br>Biosaxony Management GmbH / biosaxony e.V.<br><br>Luther Rechtsanwälte |

## 5. Navarra – European Entrepreneurial Region for Personalised Medicine

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| Project \| Initiative Title \| Logo(s) | | Navarra: European Entrepreneurial Region for Personalised Medicine<br><br>COMPREHENSIVE STRATEGY OF PERSONALIZED MEDICINE IN NAVARRA |
| Organisation name | | Government of Navarra-Gobierno de Navarra_GN |
| Country | | Spain |
| Region | | Navarra |
| Contact person | | Ángel Alonso Sánchez y Sara Torres Lizasoain |
| Contact email | | angel.alonso.sanchez@navarra.es<br>storresl@navarra.es |
| Website: | | https://gobiernoabierto.navarra.es/es/participacion/procesos/estrategia-integral-medicina-personalizada-navarra |
| Keywords: | | strategy , personalized, medicine, strategy, integral, industry, hospitals, research centers, patient, health, prevention, prediction, participative |
| Duration: | | 2020-2030 |
| Area of application | | medicine |
| DESCRIPTION | Main challenges tackled (max 200 words) | The implementation of personalized medicine in the health system requires a methodological great leap that includes:<br>• Normative and ethical modifications.<br>• Large infrastructures.<br>• Agile integration into the health system.<br>• Specialized training program at all levels (undergraduate and postgraduate) and acceptance by the public. |
| | Objectives (max 200 words) | Regional Strategy:<br>• Comprehensive Strategy of Personalized Medicine in Navarra<br>• https://gobiernoabierto.navarra.es/sites/default/files/resumen_estrategia_medicina_personalizada.pdf<br>• Conjunction of three departments:<br>• Health Department,<br>• Industry and Strategic Projects Department<br>• Innovation, Universities and Digital Transformation Department |

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| | | Infrastructures: https://www.irisnavarra.com/<br><br>• Scientific Infrastructure and Equipment Sharing System of Navarre; https://www.siessnavarra.com/<br>• Sequencing Center: https://hpc.nasertic.es/ngs/secuenciacion; https://navarracapital.es/un-nuevo-centro-para-analizar-el-genoma-humano-en-24-horas/<br>• Data Storage and interpretation center: Nasertic<br>• Electronic Medical Records<br><br>R&D: Strategic Projects:<br><br>• Genomics and Personalized Medicine Projects: (Industry & Research Centers & Hospitals) https://www.navarra.es/es/tramites/on/-/line/Ayudas-para-realizar-proyectos-estrategicos-de-I-D-en-2021-2024; https://navarracapital.es/navarra-impulsa-diez-proyectos-de-medicina-personalizada-por-19-millones/<br>• R&D projects (individual (Industry), transference (Ind+RCentres), collaboration (Ind+Ind+…+RCentres):https://www.navarra.es/es/tramites/on/-/line/Ayudas-para-realizar-proyectos-de-I-D-Convocatoria-2021<br><br>Training:<br><br>• Free taxes training: http://www.unavarra.es/sites/actualidad/contents/noticias/2021/06/22/la-upna-y-la-fundacion-instituto.html<br>• New degree in Medicine: with compulsory subjects in genomics and personalized medicine: https://www.unavarra.es/sites/grados/salud/medicina/plan-de-estudios.html<br>• Technology degrees: Biotechnology Degree, Data Science degree, Science Degree, University Expert in Data Science and Machine learning, etc. http://www.unavarra.es/sites/grados/ciencias.html<br><br>Integration of Personalized Medicine in Health System:<br><br>• Different platforms in progress<br><br>Collaborative Network:<br><br>• Dynamization web “DNa MedPer” (among agents from the Industry Technology Centers, Patient’s associations, etc. https://medicinapersonalizada.navarra.es/es/ |

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| | | Support to start ups in health through:<br>• scale up programs, training, internalization plans, infrastructures: https://www.cein.es/crea-tu-empresa/emprendimientoensalud/ |
| | Main concept and methodologies involved (max 200 words) | The development of the Comprehensive Strategy for Personalized Medicine of Navarra for the period 2020-2030 was carried out by means of the constitution, at the proposal of the Interdepartmental commission, of a Technical Committee that performed the functions of coordination and advice throughout the project.<br>The action plan proposal was formulated through the constitution of different Groups of Work (GT), coordinated by the Technical Committee, made up of experts and professionals relevant aspects of the different health areas involved in the development of Personalized Medicine.<br>Other complementary profiles as managers of international health innovation projects, scientific-technological directors of health research institutes, responsible for technology and research centers biomedical, hospital managers, patient associations, responsible for development of R&D&I policies of Autonomous Communities, etc.<br>The supervision, review and validation of the documentation generated was carried out by the Interdepartmental Commission composed of the heads of the different departments of Government of Navarra involved in the implementation, advancement and development of Personalized Medicine in the Autonomous Community. |
| | Impact (health, scientific, industrial, socio-economic or others enabled by the project/initiative (max 200 words) | • Economic development of the health sector and the personalized medicine subsector.<br>• Repercussions on job creations and returns in the form of tax contributions.<br>• Represents an opportunity for cross-sector development and cooperation.<br>• Identifying and positioning our region as a model of efficient and quality healthcare.<br>• Increases the responsibility and social awareness of well-being of our citizens. |
| | Funding and Investment (please specify the source: public, private, Structural or other types of funds) | Regional funds |
| | Key stakeholders involved | Industry, Research Centers, Hospitals, etc. |

## 6. Onkolotse – a Personal Guide for Cancer Patients

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| Project \| Initiative Title \| Logo(s) | | Onkolotse (Cancer guide)<br>onkolotse<br>Sächsische Krebsgesellschaft e.V. |
| Organisation name | | Sächsische Krebsgesellschaft e.V. (Saxon Cancer Society) |
| Country | | Saxony/Germany |
| Region | | Germany |
| Contact person | | Dr. Ralf Porzig |
| Contact email | | info@onkolotse.de |
| Website: | | www.onkolotse.de |
| Keywords: | | cancer, cancer guide, personal support, adherence |
| Duration: | | Project started in 2010 |
| Area of application | | Oncology |
| DESCRIPTION | Main challenges tackled (max 200 words) | Wide range of Challenges for our current health system:<br>• demographic development<br>• increasingly complex therapeutic setting<br>• participative approaches to decision making (idea of informed patients)<br>• non-compliance = negative effects on therapeutic success plus huge pharma-economic effects<br>• Studies in Germany estimate direct/indirect costs of non-compliance of about 7,5 - 10 bn €<br><br>Modern therapy: Move from compliance to adherence.<br><br>The cross-sectoral personal support of Onkolotsen for cancer patients and relatives along the treatment path<br><br>- offers guidance and support in an increasingly complex therapeutic setting,<br>- can improve patient information level, help them to find their way in the health care system, and enables them to truly participate in the decision process,<br>- can improve doctors time management, therapy success, adherence and reduce non-compliance cost,<br>- can provide a wider service range and better services for oncological |

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| | | patients and improve communication with patients on therapy, medication and quality of life,<br>- can improve patient satisfaction and medical/care-team effectiveness and efficiency. |
| | Objectives (max 200 words) | Improve the personal support of cancer-patients and their relatives along patient's treatment path and across all medical sectors in Germany which normally do not work together frictionless (Idea: One face to the patient). Onkolotsen (= Cancer guides) will help patients and their families<br>• to find their personal way through cancer treatment,<br>• to become an informed patient,<br>• to improve compliance and coping and<br>• to help them to live with the disease and to make the best out of their life. |
| | Main concept and methodologies involved (max 200 words) | The Onkolotse is a personal support offer for cancer patients which provides guidance and support in the increasingly complex therapeutic setting in the German health care system. Building on patient education and trust it helps patients to cope with their illness. The offer is based on a standardized training program (incl. role plays, case studies, teamwork, expert presentations) for nurses and other medical care personal involved in coaching and supporting cancer patients and their relatives combined with an ongoing training and support after successful completion of the training program. |
| | Impact (health, scientific, industrial, socio-economic or others enabled by the project/initiative (max 200 words) | 250 cancer guides were trained (2010-2021) on the basis of an official training curriculum. They help patients and their relatives to find their way through cancer treatment and into a new life, e. g. in university hospitals and cancer centers in Dresden, Leipzig, Chemnitz, Greifswald, Erlangen, München and Halle/Saale as well as in doctor's offices in Dresden, Frohburg, Neumünster und Rodgau or pharmacies in Werdau, Leipzig and Chemnitz. Training courses take place on a continuous basis in Saxony as well as in other regions in Northern Germany. 2 successful evaluation programs of the approach – in cooperation with health insurance companies - finished in 2021 (Innovation fund program). An additional clinical trial of Saxon Cancer Society is still underway. |
| | Funding and Investment (please specify the source: public, private, Structural or other types of funds) | The initial pilot projects were supported by the Free State of Saxony and the European Union (2010-2012 and 2012-2014 / public support). As of 2013 the training courses are offered on a commercial base and paid by participants or hospitals, cancer centers, doctor's offices, pharmacies and cancer help desks for their employees. The ongoing support of cancer guides is funded by Saxon Cancer Society based on e.g. donations. |
| | Key stakeholders involved | Sächsische Krebsgesellschaft e.V. (Saxon Cancer Society) |

## 7. TREAT-NMD Global Registry Network

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| Project \| Initiative Title \| Logo(s) | TREAT-NMD Global Registry Network |
| Organisation name | TREAT-NMD Alliance Ltd |
| Country | International |
| Region | International / worldwide |
| Contact person | David Allison |
| Contact email | info@treat-nmd.com |
| Website: | https://treat-nmd.org/patient-registries/what-are-the-treat-nmd-global-registries/ |
| Keywords: | Patient registries, neuromuscular disorders, FAIR principles |
| Duration: | 2007-on |
| Area of application | Neuromuscular disorders (NMD) |
| DESCRIPTION — Main challenges tackled (max 200 words) | TREAT-NMD is a network of excellence for the NMD field that provides infrastructure to ensure that the most promising new therapies reach patients as quickly as possible. Established in 2007 as an EU funded 'network of excellence', its focus has been on the development of tools that industry, clinicians and scientists need to bring novel therapeutic approaches into the clinic, and on establishing best-practice care for patients living with a NMD condition.<br><br>Recent years have seen rapid developments in the NMD field with promising preclinical results and new therapies already approved or approaching the market. Yet therapy development for rare NMD still faces several barriers for patients, clinicians and researchers and pharmaceutical industry. Particularly, a lack of implemented, standardised care guidelines prevent many from receiving optimal care. Support tools such as validated clinical outcome measures, data collection guidance and standard operating procedures for research protocols may hold back therapeutic development. For diseases with therapy options available, standardised and available data on the safety and efficacy of the different therapies are needed for informed decision-making. For post-marketing commitments of industry, establishment of phase 4 studies often places an unfeasible burden on the data providers and leads to drug-specific data silos. |

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| | Objectives (max 200 words) | To standardise and harmonise collection of NMD clinical data to support the development of new treatments and intervention, improve safety and efficacy assessment of existing treatments, give a better understanding of disease progression and improve standards of care for people living with NMD. |
| | Main concept and methodologies involved (max 200 words) | The TREAT-NMD Global Registry Network is also known by the legacy name 'TGDOC' (TREAT-NMD Global Data systems Oversight Committee), its governing board includes representatives from all participating registries and patient representatives. The TGDOC is governed by a charter and Standard Operating Procedures (SOPs) which outline the relationship among the individual registries and with third parties (such as industry and academic partners wanting to enquire into the Global Registries Network for research or clinical trials). A dedicated Executive Board leads on the strategic activities and manages operations.<br><br>Collection of the TREAT-NMD Core Datasets allows standardisation of data across a variety of registries (national/international, clinical/patient entered data, etc.) according to the FAIR (Findable, Accessible, Interoperable, Reusable) principles. Core datasets have been established for the main NMD disease (groups) and are periodically revised through a consensus process which involves all main stakeholders to keep data collection aligned with the most updated clinical developments in the specific fields.<br><br>Data from participating registries have the option to be combined into a Central Data Warehouse either directly through the TREAT-NMD Global Registries Platform (GRP) infrastructure, which registries can use either directly to host their patient data, or indirectly, where registries embed the core dataset into their own system and share data with TREAT-NMD via the GRP. |
| | Impact (health, scientific, industrial, socio-economic or others enabled by the project/initiative (max 200 words) | The harmonisation of datasets enables TGDOC to run enquiries into patient data with reassurance that it is of a high quality and standardised throughout the network. Up to 2021, 40 enquiries have been completed for use by pharmaceutical companies in the NMD field, resulting in feasibility enquiries for clinical programmes, clinical trial planning, recruitment support for clinical trials and multinational studies focusing on health economics. This work is undertaken with the aim of ensuring that promising new therapies can be brought to patients as quickly as possible.<br><br>Real-world data from the TREAT-NMD Global Registries Network may also significantly contribute to:<br><br>1. improved understanding of disease progression in treated and untreated patients<br>2. long term evaluation of safety and efficacy for existing or new treatments |

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| | | 3. clinical trial design and outcome measure selection<br>4. facilitate dissemination of better standards of care for people living with NMD.<br>The TREAT-NMD Global Registry Network activity has also greatly contributed to share good practice and FAIR principles on patient data stewardship and management. In 2016, the TREAT-NMD Global Registries Network received endorsement from the International Rare Diseases Research Consortium (IRDiRC) as a Recognized Resource. |
| | Funding and Investment (please specify the source: public, private, Structural or other types of funds) | Local activity of the individual registries is typically supported by institutional funds to clinicians and/or by Patient Organisations. Additional contribution may derive from specific agreements for data use with pharmaceutical industries either local or through TGDOC. The TREAT-NMD infrastructure (GRP platform, TGDOC management, development of the core datasets) received unconditional support from pharmaceutical industry and is supported by fees for service for TGDOC enquiries. |
| | Key stakeholders involved | Patients and Patient Organisations; clinicians; pharmaceutical industry |

## 8. TRON – Translationale Onkologie an der Universitätsmedizin der Johannes Gutenberg-Universität Mainz gemeinnützige GmbH

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| Project \| Initiative Title \| Logo(s) | |  |
| Organisation name | | TRON – Translationale Onkologie an der Universitätsmedizin der Johannes Gutenberg-Universität Mainz gemeinnützige GmbH |
| Country | | Germany |
| Region | | Rheinland-Pfalz |
| Contact person | | Andrée Rothermel |
| Contact email | | Tron-pm@tron-mainz.de |
| Website: | | www.tron-mainz.de |
| Keywords: | | Cancer, Oncology, Biomarkers, Vaccines, Immuno-Oncology, Clinical Translation, Personalised Medicine, Innovative Therapies |
| Duration: | | TRON was founded in 2010 by Ugur Sahin, Özlem Türeci and Christoph Huber |
| Area of application | | Translational Oncology, Personalised Medicine |
| DESCRIPTION | Main challenges tackled (max 200 words) | TRON gGmbH – Translational Oncology at the University Medical Center of the Johannes Gutenberg University in Mainz (Germany) is a trans-disciplinary, not-for-profit research organization. Specializing in cancer immunology and genomics, our research teams identify and characterize disease-relevant molecular targets, including developing tailored bioinformatical and novel biostatistical approaches. We design and improve cutting-edge technologies for biomarker discovery and biopharmaceutical drug development. Once we establish preclinical proof-of-concept, we aim to translate our results into (clinical) patient care via cooperation partners. Several TRON inventions have found their way into clinical trials. In addition, TRON technologies have been integrated in BioNTech´s Comirnaty® vaccine. |
| | Objectives (max 200 words) | TRON pursues basic and applied research in oncology and immunology to develop innovative new drugs and diagnostics for the treatment of cancer and other severe diseases of the immune system with a particular focus on personalized therapies and biomarkers. |
| | Main concept and methodologies involved (max 200 words) | Personalized medicine, in which physicians tailor treatment to specific patients, has high potential for increasing treatment efficacy and lowering the costs of drug development and medical treatment. TRON's research of novel biomarkers is a key contribution to this area, as biomarkers are a key tool for designing |

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| | | treatment plans and are used to address defined characteristics in combination with targeted therapeutics. Additionally, TRON is specialized in all technologies and techniques for profiling clinical specimens, including the next generation high-throughput sequencing platform, data mining and the analysis of basic and clinical study results.<br><br>In addition, TRON offers the following expertise:<br><br>- Characterization of Immune Therapeutics<br><br>- Therapy Studies in Mouse Models<br><br>- Immunogenicity Studies in Mouse Models<br><br>- In vivo Imaging<br><br>- Data Analysis<br><br>- Biostatistical Analysis<br><br>-Systems Biology and Computational Immunology |
| | Impact (health, scientific, industrial, socio-economic or others enabled by the project/initiative (max 200 words) | TRON conducts collaborative research into immunological mechanisms and therapeutic modulation of the immune system. Cutting-edge research and applied studies are combined to elucidate immune mechanisms of disease and to modulate immunity in autoimmune diseases, allergy, cancer and infectious diseases. Although TRON was founded in 2010, several TRON inventions are already undergoing clinical testing and may lead to improved patient care in the next few years. In addition, TRON technologies were used to develop BioNTech´s Comirnaty® vaccine. As such, TRON inventions have had major health, scientific, industrial and socio-economic impact. |
| | Funding and Investment (please specify the source: public, private, Structural or other types of funds) | The research at TRON gGmbH is funded by national (DFG, BMBF) and international (EU) funding initiatives. For this, TRON is taking part in several local, national and international research consortia. In addition, TRON is partnering with biotech companies and pharmaceutical industries. |
| | Key stakeholders involved | TRON partners with medical universities, research institutions in the field of genomics, immunology and/ or oncology, biomarker diagnostics developing companies, drug developing biopharmaceutical companies, scientists and researchers with an interest in translation. |

## 9. SaxoCell – Living Drugs – Precision Therapy Cluster for Saxony

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| Project \| Initiative Title \| Logo(s) | | SaxoCell® |
| Organisation name | | SaxoCell core partners:<br>Fraunhofer Institut für Zelltherapie und Immunologie IZI<br>Technische Universität Dresden<br>Universität Leipzig<br>Klinikum Chemnitz gGmbH |
| Country | | Germany |
| Region | | Saxony |
| Contact person | | Dr. Thomas Tradler, MBA |
| Contact email | | thomas.tradler@izi.fraunhofer.de |
| Website: | | www.saxocell.de |
| Keywords: | | Cell and gene therapy; living medicine, manufacturing |
| Duration: | | 01.10.2021-30.09.2024 |
| Area of application | | Cell and gene therapies |
| DESCRIPTION | Main challenges tackled (max 200 words) | In Germany, medicine of the future faces the societal challenge of providing people with innovative care that is accessible to all but also affordable. The central goal of the SaxoCell cluster of the future is therefore to develop state-of-the-art cell and gene therapeutics, so-called "living medicines" for many patient groups, which are highly efficient and available at low cost. The aim is to produce the patient's own (autologous) or foreign (allogeneic) immune or stem cells with precise functions and at socially acceptable costs on an industrial scale and make them clinically available to patients as part of a personalized therapy concept. Through the regional cooperation of academic and clinical institutions, research institutes, start-ups and larger industrial partners, health authorities and ethics committees, an attractive economic model with high regional, but ultimately also national value creation potential is to be created in Saxony. |

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| | Objectives (max 200 words) | • Development of novel ATMPs (advanced therapy medicinal products) and expansion of their previous areas of application<br>• Improving the tolerability and efficacy of ATMPs through the latest cell and gene therapy (CGT) technologies<br>• Increasing manufacturing efficiency through automation / artificial intelligence (AI) and standardization<br>• Creation of an innovative preclinical and clinical environment for the optimal development of the industry in Saxony, also through measures of professional education and training<br>• Strengthening regional, national and international technology transfer by attracting investments and innovations from international companies to Saxony |
| | Main concept and methodologies involved (max 200 words) | SaxoCell® has a strong focus on innovative R&D. A comprehensive search for attractive cell and gene therapy projects was conducted. The selection of the applications received was based on, among other things, an assessment of the level of innovation, the industrial feasibility and applicability, the industry contribution already acquired to the project, and the hoped-for positive impact on the expansion of the regional industrial sector. Eventually, twelve R&D projects were selected which address current limitations in the field of novel ATMPs, while providing solutions to regulatory and health economic aspects. In addition, the cluster established several cross-project technical and clinical platforms to support individual R&D projects, addressing special R&D project needs like clinical trial management, Omics technologies, manufacturing automation and others. Moreover, SaxoCell also established the central management & communication hub SaxoCellHUB, which works in a cross-project manner as well, creates and maintains an innovation oriented cluster culture – and takes care of R&D supporting activities like project and stakeholder management, transfer, PR, cluster marketing and other tasks. |
| | Impact (health, scientific, industrial, socio-economic or others enabled by the project/initiative (max 200 words) | SaxoCell® will significantly improve regional networking in Saxony in the field of cell and gene therapies, will integrate further partners and initiatives nationwide and aims at closing corresponding gaps in the cell and gene therapy value chains. In this way, synergy potentials will be raised and realized. In addition, the cluster will massively increase the visibility of the activities of Saxon and nationwide partners in the field of cell and gene therapy – both at the level of research and with regard to the R&D activities of industrial partners. Thus, access to funding will be facilitated and expanded for the players, and structures will be created to accelerate clinical implementation (network of clinical players and cooperation with regulatory authorities). Through these and other activities, SaxoCell® will thus significantly increase the attractiveness for investments of national and international companies in this industrial sector and contribute substantially to the development and further strengthening of an innovative cell and gene therapy industry in Saxony, Germany. |

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| | Funding and Investment (please specify the source: public, private, Structural or other types of funds) | SaxoCell is funded from BMBF clusters4future initiative as well as own contribution from industry partners involved in several R&D projects for 3 years with an amount of approximately 14 Mio. € with an option to extend the project for a total of up to 6 more years. |
| | Key stakeholders involved | • Fraunhofer Institute for Cell Therapy and Immunology IZI, Leipzig<br>• Technische Universität Dresden<br>• Universität Leipzig<br>• Klinikum Chemnitz gGmbH<br>• City of Leipzig<br>• City of Dresden<br>• City of Chemnitz<br>• Free State of Saxony |

## 10. SaxoChiLD – Saxonian Child health innovation Leipzig - Dresden

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| Project \| Initiative Title \| Logo(s) | SaxoChiLD<br><br>Network between Leipzig and Dresden for the DZKJ application funded by the BMBF (Deutsches Zentrum für Kinder und Jugendmedizin)<br><br>SaxoChiLD – Saxonian Child health innovation Leipzig - Dresden |
| Organisation name | SaxoChiLD |
| Country | Germany |
| Region | Saxony |
| Contact person | Prof. Dr Antje Körner |
| Contact email | antje.koerner@medizin.uni-leipzig.de |
| Website: | https://saxochild.de/ |
| Keywords: | Child, youth, health, German Center, network, obesity, environment, immunology, mental health |
| Duration: | September 2021 – tbd (currently in a concept development phase till February 2022 with 6 other partner sites in Germany for the main proposal). Official start date of DZKJ would be January 2023. |
| Area of application | Pediatrics, basic research, clinical research, child and youth health |
| DESCRIPTION<br>Main challenges tackled (max 200 words) | Over the past century, biomedical research and public health efforts have made tremendous advances in containing child morbidity and mortality and improving child health and<br><br>development. This has been promoted by industrial, technological and societal transformations. Now, some of these environmental changes partially offset advances in<br><br>child health by imposing new health challenges that give rise to chronic disorders. These changes also influence the interplay between psyche and soma, modulating the expression<br><br>of somatic and mental health problems. The period from conception through birth and childhood to adolescence comprises particularly vulnerable phases of development, each<br><br>with sustained and life-long consequences on health. Thus, the interaction of child health and development with the environment needs to be |

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| | | comprehensively addressed. Although many environmentally triggered diseases are diagnosed in adulthood, they often originate in childhood or even the prenatal phase on the background of an individual predisposition. This is particularly relevant to chronic metabolic, inflammatory, and mental disorders. Yet, neither the risks nor the emerging diseases associated with our continuously changing environment have been sufficiently addressed to improve preventive and therapeutic strategies. |
| | Objectives (max 200 words) | In an overarching approach, SaxoChiLD combines comprehensive epidemiological surveillance of child health and development in a dynamic environmental context with special consideration of the psyche-soma interaction. SaxoChiLD provides the entire interdisciplinary spectrum of scientific and clinical excellence in pediatric epidemiology, environmental research, immunology, infectious diseases, obesity and mental health along with a powerful complementary infrastructure, including an internationally unmatched collection of cohorts.<br><br>We address new health risks emerging from the interplay between predisposition and our changing environment, and we identify risk and resilience factors as well as underlying mechanisms. With focus on obesity, immunopathies and mental disorders, SaxoChiLD implements the full translational chain from epidemiological studies to basic and clinical science discoveries to the development and application of novel targeted and more precise detection, prevention and therapy tools. Amenable to other entities, this holistic approach leads to a conceptual change in pediatric medicine towards promotion of child health on both, a population and patient-tailored level, particularly, but not only for civilization diseases originating in childhood.<br><br>During the current concept development phase the proposal of SaxoCHiLD will now be refined together with the other 6 national partner sites. The aim is to form a new German Center for Child and Youth Health. |
| | Main concept and methodologies involved (max 200 words) | The main concept is to combine regional and national expertise as well as infrastructure to address the above-mentioned challenge via a translational research approach.<br><br>SaxoChiLD's major contribution will be:<br><br>- Infrastructure for patient care and education of health care professionals<br>- Epidemiological expertise, cohorts, registries, and biobanks<br>- Environmental monitoring<br>- Expertise on the innate immune system to infection associated inflammatory disease, autoinflammation, and autoimmunity<br>- Expertise in metabolism and childhood obesity<br>- Contribution to neuro-psychosocial aspects to child health, development, and disorders |

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| | | - Facilities, core units, and platforms for experimental biomedical research<br>- Information technology<br>- Institutional commitment for pediatric research |
| | Impact (health, scientific, industrial, socio-economic or others enabled by the project/initiative (max 200 words) | The impact will be mainly scientific and research results can be transferred to the public and patient care.<br><br>One could determine new risk and resilience factors, determine the most critical vulnerable age periods and derive indicators of potential mechanisms of civilization diseases originating in childhood. New results can lead to better understanding of mechanisms, novel therapies, prevention guidelines, and policy making. Our approach might result in a conceptual change in pediatric medicine towards promotion of public health on a populational as well as patient-centered individual level, particularly but not restricted to civilization diseases. |
| | Funding and Investment (please specify the source: public, private, Structural or other types of funds) | BMBF – Federal Ministry of Education and Research; SMWK – Saxon State Ministry Science, Culture and Tourism |
| | Key stakeholders involved | University of Leipzig; University of Leipzig Medical Center; Technical University Dresden; University Hospital Carl Gustav Carus, Dresden; Helmholtz-Centre for Environmental Research – UFZ, Leipzig; Max Planck Institute for Evolutionary Anthropology, Leipzig; Robert Koch Institute, Berlin |