

Charité Research Organisation

Our Concept

Charité Research Organisation

Charité owned | operationally independent | scientifically driven



Qualified Phase 1 and POC unit

30 beds (expanding to 70 in early 2018) with an additional out-patient facility. Access to patients, clinical expertise, biomarker, imaging and diagnostic capabilities. Independent subsidiary of Charité – Universitätsmedizin Berlin.



Staffing

Approximately 150 dedicated staff, including board certified physicians, nurses, lab technicians, pharmacists, PMs, QMs, data entry staff and independent QA.



Experience

Conducted more than 300 First Time in Human, Phase 1 and POC projects since launch in 2006. Pioneered FTIH and Phase 1 studies in patients.



Key research areas

Rheumatology, dermatology, gastroenterology, endocrinology, pulmonology, cardiology, neurology, psychiatry, infectious diseases, ophthalmology, oncology.



Study types

First Time in Human, Phase 1, POC. Includes biosimilar studies, imaging and methodology studies, blood and tissue sampling studies, pre-screening studies.



Access to accredited safety lab, ISO certified biomarker labs, imaging facilities and licensed GMP pharmacy.

CRO can provide a full service

Internalised

Scientific & Regulatory Consultancy

Project Management

Protocol Development

Ethical & Regulatory Submissions

Clinical Conduct

Medical Writing

Partnered (University or external)

Routine Laboratory Analysis

Biomarker Validation & Analysis

GMP Pharmacy

Monitoring

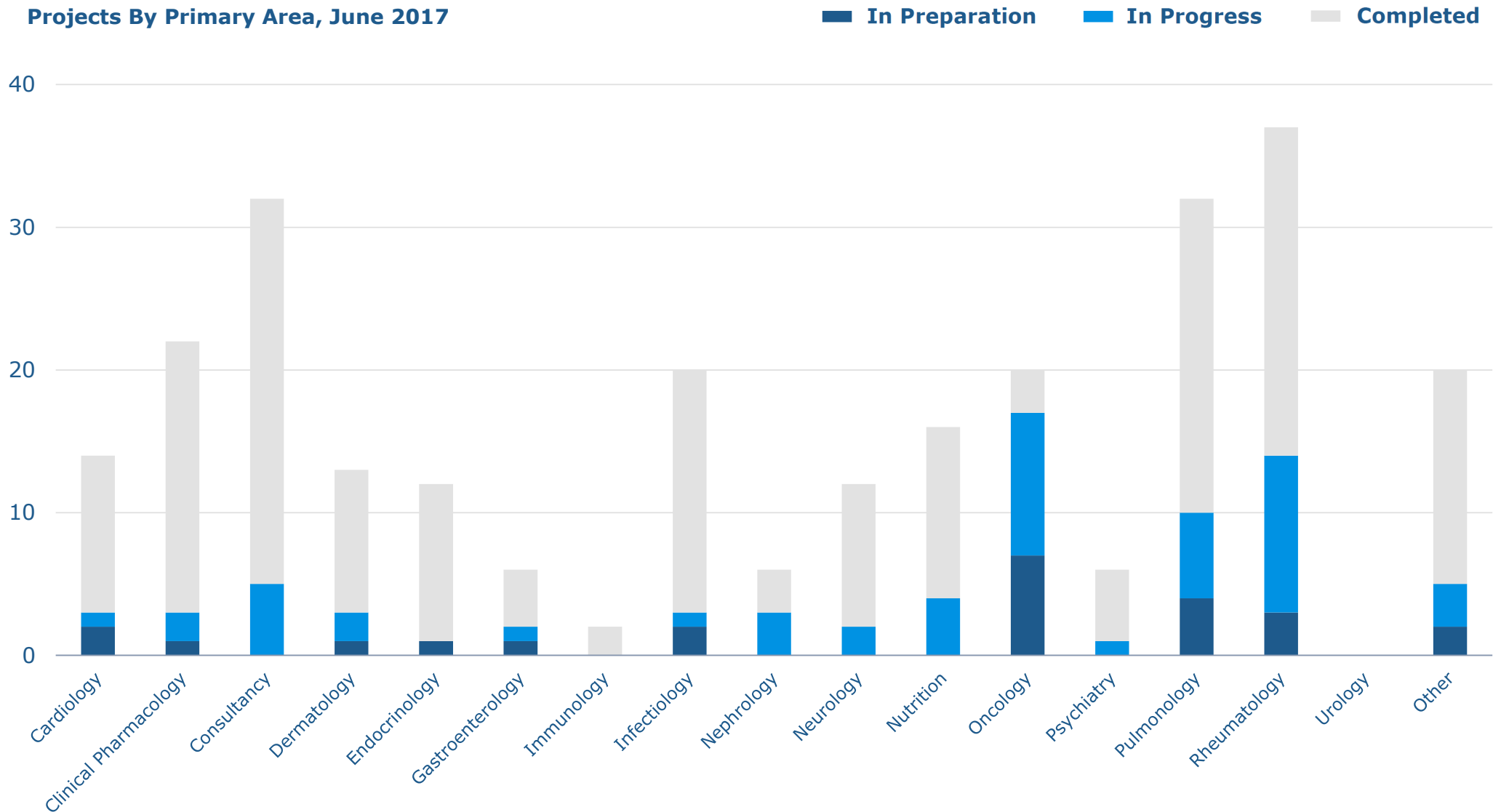
Data Management

Statistical Analysis

Overseen by an ICH-GCP compliant QA / QM system

325 FTIH-PoC Projects to date

From more than 50 industry sponsors



Charité Research Organisation

FTIH-POC Success Stories

Phase I in HV and Crohn's Patients

- ▶ SAD study in n=64 HV patients & n=24 Crohn's patients
- ▶ Completed with a single centre approach

Phase I in Moderate-Severe Psoriasis Patients

- ▶ Multiple dose interaction study in n=21 moderate-severe psoriasis patients
- ▶ German-wide recruitment and enrolment completed in approx. 4 months
- ▶ Completed with single centre approach

FTIH in RA Patients

- ▶ Study in n=92 RA patients across SAD i.v., SAD s.c. and MAD s.c. parts with imaging and biomarkers analysed locally
- ▶ Completed with a single centre approach

Phase I in HVs and Asthma Patients

- ▶ Study with n=80 HVs across MAD and FE parts and n=54 mild-moderate asthma patients enrolled in approx. 2 months
- ▶ Completed with a single centre approach

Recruitment Case Study

Phase I Psoriasis

Study Population

Psoriasis

Area

Dermatology

Assessments

PASI, psoriasis target lesion score

Recruitment Strategy

Database & online marketing

Single-Centre

n=61 psoriasis patients in 6 groups

Study Duration

4 weeks SCR
12 to 17 weeks treatment + FUP

Total Enrolment Duration

Approximately 8 months of active enrolment across all groups

Screened

156

Randomised

61

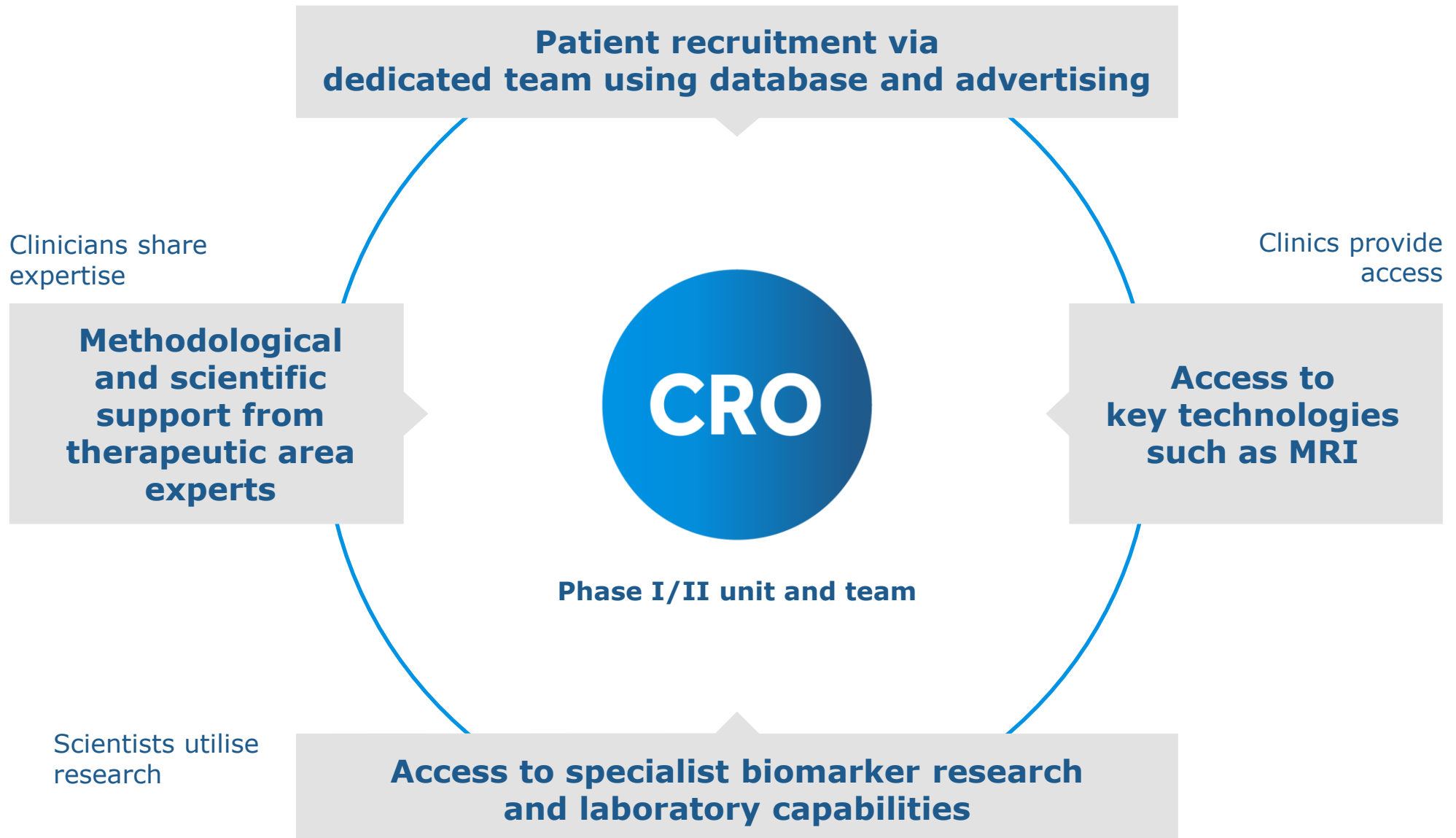
Completed

58

3 drop-outs

- ▶ 40 out of 61 patients [66%] randomized with a PASI score of 4 or higher on Day -1
- ▶ Average PASI score of 8,3 on Day -1 for randomised patients
- ▶ Average recruitment rate of 8 per month

CRO Centres of Excellence Approach



CRO Centres of Excellence

**Clinical and scientific support
across multiple disciplines**

**Key examples include
dermatology and rheumatology**

**Prof. Dr. med.
Gerd Burmester**

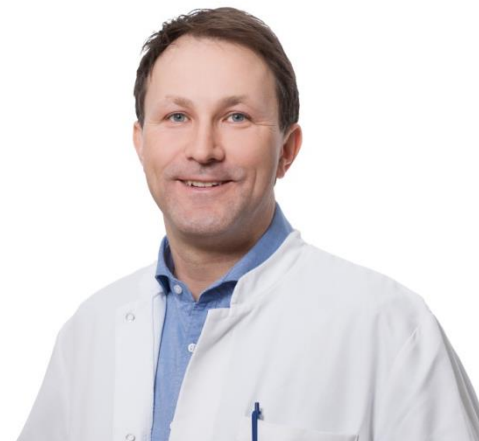


Prof. Dr. med. Gerd Burmester

**Imaging with central scoring
services: MRI, US, FOI, X-Ray,
CT, PET**

**Prof. Dr. med.
Marina Backhaus**

**PD Dr. med.
Kay-Geert Hermann**



PD Dr. med. Kay-Geert Hermann

**ISO certified Immunological
Study Laboratory (ISL)**

**Off the shelf assays, transfer
and validation of sponsor
assays, research laboratory**

**Prof. Dr. med.
Hans-Dieter Volk**

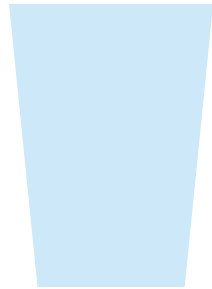
PD Dr. Gerald Grütz



Prof. Dr. med. Hans-Dieter Volk

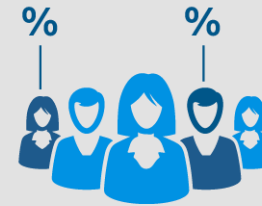
Feasibility Process

Determining the number of potentially eligible subjects



Prevalence

What is the general indication specific prevalence?



- ▶ Prevalence of rheumatoid arthritis: approximately 0.5%



Subset of Population

Based on the prevalence what is the subset of the Berlin/German population we can target?



- ▶ Population of Berlin/Germany: 3.5/81 million
- ▶ RA patients in Berlin/Germany: $3.5/81 \text{ million} \times 0.5\% = 17,500/405,000$



Potentially Eligible Subjects

Based on experts' judgement what percentage of this subset is represented by the defined inclusion/exclusion criteria?



- ▶ Percentage of RA patients represented by inc./exc. criteria: 30% (for example)
- ▶ Potentially eligible RA patients in Berlin/Germany: $17,500/405,000 \times 30\% = 5,250/121,500$

CRO Recruitment Approach



**Appeal directly
to the patient**



**Have the resources
to treat the patient
with utmost respect**



**Financially
compensate the
patient appropriately**

Innovation: A National Single Centre

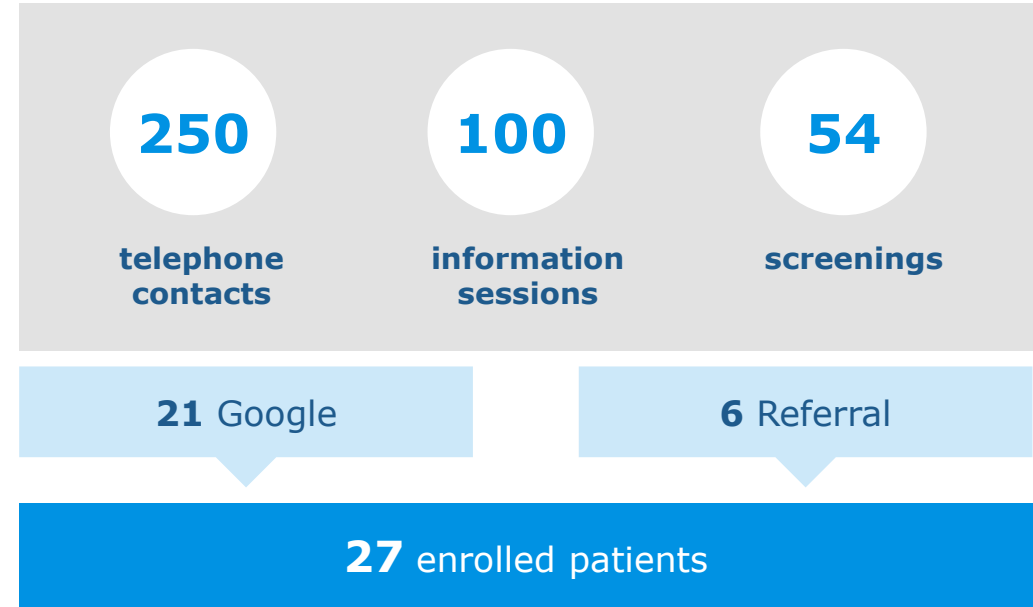
One centre | German-wide Sjögren Patient Recruitment

11 patients from Berlin
16 from the rest of Germany

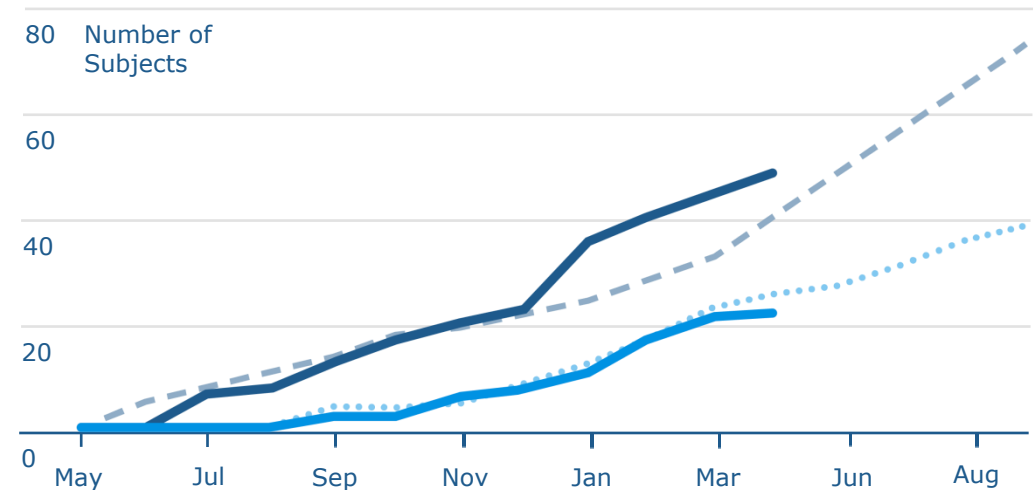


Planned Screening ---
Actual Screening —

Planned treated ---
Actual treated —



Study VAY736X2201 recruitment



Innovation: Pre-Screening Studies

**Pre-screening study
approved by local ethics**

**n=244 asthma patients identified and pre-screened against
key study inc./exc. criteria before and during conduct of HV part**

**Core block of patients
identified to push
to main study**

n=108 considered to be “probably” eligible for main study

**Ultimately n=98 retained
for main study screening**

n=98 entered into main study screening

n=54 randomised

**n=54 randomised in just over 2 months in
2 parallel cohorts of 27**

CRO Centres of Excellence

“Working with Charité Research Organisation allows me to contribute both scientifically and practically. We have been able to incorporate innovative MRI imaging techniques in early phase RA studies – such as Dynamic Contrast Enhanced MRI – and also explore the potential for MRI to be utilised in other indications such as OA and SpA.”

PD Dr. med. Kay-Geert Hermann
Charité Radiology Institute





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