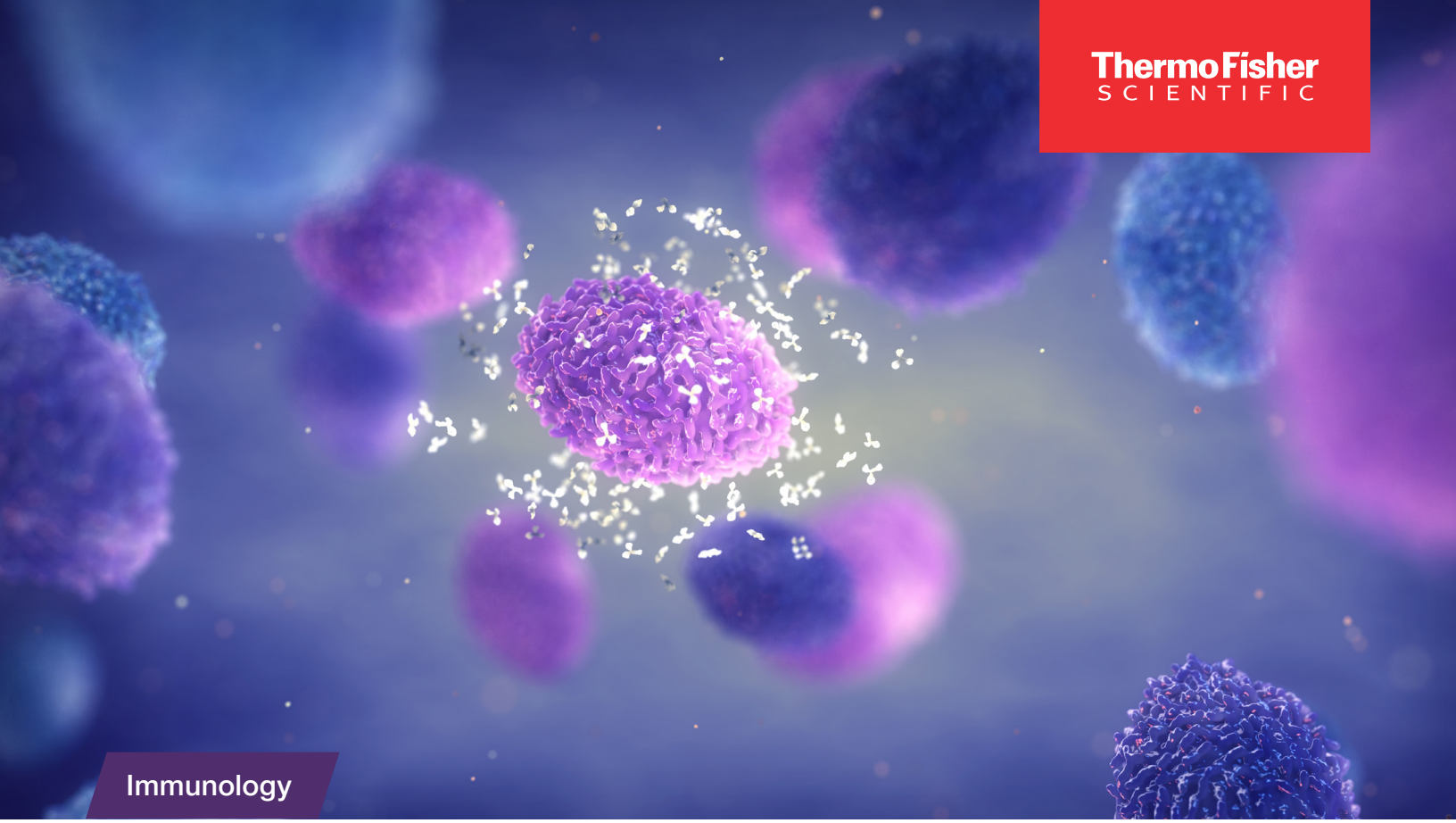


Bring rare immunology therapies to patients sooner with our integrated scientific and clinical trial expertise

Disease background information

Rare immunology refers to a group of uncommon, often genetic disorders of the immune system characterized by immune deficiency, immune dysregulation, or pathologic inflammation.

Effectively treating rare immunology disorders depends less on one single therapy and more on **getting the right diagnosis early and matching treatment precisely to the immune defect.** Although care has improved substantially over the past two decades, delayed diagnosis and fragmented referral pathways still contribute to preventable morbidity and mortality.

The focus of the PPD™ clinical research business of Thermo Fisher Scientific is to enable sponsors to advance patient-centered development strategies that reflect the needs of patients, caregivers and specialist physicians.

Challenges with rare immunology clinical research

Why rare is different

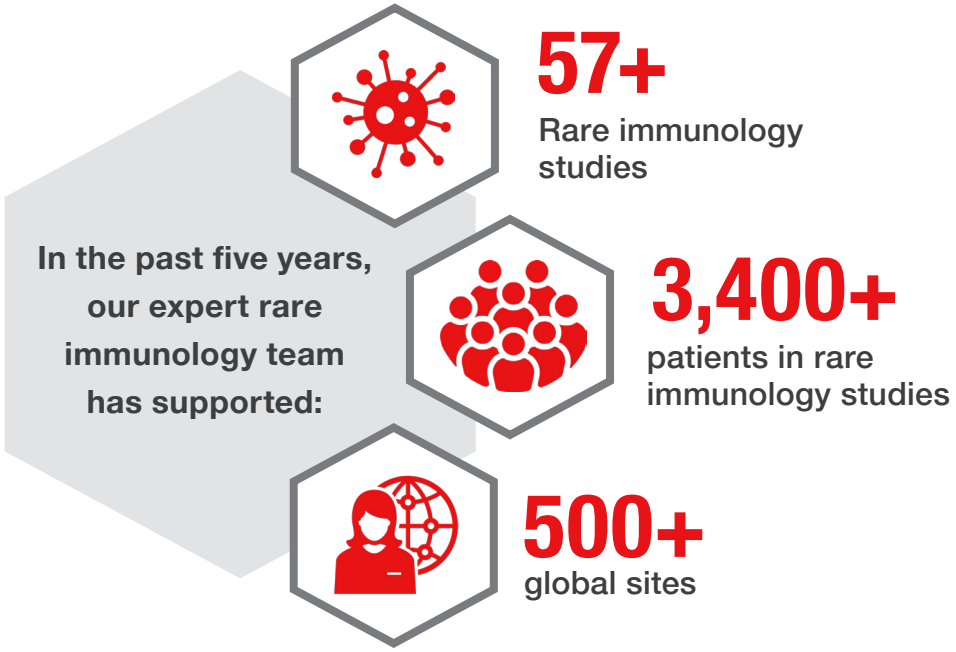
Rare immunology development presents distinct scientific and operational challenges that require a specialized approach.

- Small, geographically dispersed patient populations requiring strong referral pathways
- Complex diagnostic criteria and delayed diagnosis timelines
- High screen failure rates and narrow inclusion/exclusion criteria
- Limited natural history data and evolving standards of care
- Need for global activation to reach adequate enrollment
- Close collaboration with patient advocacy groups and specialty centers

Success in rare immunology trials depends on deep therapeutic expertise, established site relationships and flexible, patient-centric delivery models.

We are the leader in key rare immunology indications

Benefit from many years of rare immunology experience, spanning all clinical trial phases and a broad spectrum of different indications.



With a more than **8,300 experienced professionals**, our immunology team brings scale, expertise, and a proven track record of successfully delivering programs across multiple rare immunology indications, including:

Indications	No. of protocols	No. of sites	No. of patients
Polymyalgia rheumatica (PMR)	1	23	26
Giant cell arteritis (GCA)	2	50	70
***Systemic sclerosis (SSc)**	17	409	845
***Immunodeficiency (all types)**	12	14	280
Hereditary angioedema	8	268	887
***Sjogren's syndrome**	6	563	851
***Dermatomyositis**	2	142	172

*Including CAR-T and T cell engagers (TCE)

Our experience and expertise

Our commitment to excellence

We are dedicated to advancing rare immunology development through:

Therapeutic knowledge:

- Solid operational experience of conducting more than 57 rare immunology clinical trials globally in the past five years
- Operational subject matter experts in cell therapy in autoimmunity, lupus, rare immunology and arthritis
- Team of programmers and specialist data reviewers who can perform targeted eligibility review and real time review of efficacy data
- A global group of seven board certified rheumatologists

Investigational sites network:

- Deep, established relationships with key opinion leaders and investigators worldwide
- Our global **PPD Select** site partnership program, designed to foster holistic, trusted, and site-centric relationships across **42 networks in 27 countries**
- Together with **Trialmed**, we offer flexible, scalable clinical trial solutions aligned to sponsor needs and budgets, with access to a best-in-class site network across **Phases I–IV**

Bring rare immunology therapies to patients sooner with our integrated scientific and clinical trial expertise

ppd.com/therapeutic-expertise/immunology/

Your trusted partner in rare immunology clinical development

What differentiates our rare capabilities

Our experience encompasses strategic consultancy from pre-clinical, analytical, regulatory, and clinical development submission for sponsors worldwide, as well as execution of various phases of clinical trials.

Specialized therapeutic leadership

- Operational subject matter experts in rare autoimmunity, rheumatology and cell therapy
- Board-certified rheumatologists providing medical oversight and protocol insight
- Dedicated rare disease recruitment experts who design and execute precision enrollment strategies tailored to the distinct realities of each rare immunology indication

Established rare referral networks

- Backed by a global site network spanning more than 40 countries, with focused activation of high-performing rare immunology centers
- Direct relationships with key opinion leaders and rare specialty centers

Patient-centric rare trial delivery

- Decentralized digital solutions including eConsent, eCOA, televisits and wearables
- Direct to/from patient shipments and home health nursing for support with home study visits or drug administration
- Active collaboration with patient advocacy organizations to increase patient engagement by incorporate patients' voice
- Optimize trial design, removing barriers to patient participation and reducing patient burden to support recruitment of diverse patient cohorts

Multidisciplinary operational execution for rare trials

Rare immunology trials often extend beyond rheumatology, requiring coordinated input across multiple specialties. We support complex studies in multisystem diseases such as systemic sclerosis and IgG4-related disease by aligning pulmonology, nephrology, dermatology, gastroenterology, and vascular expertise across global site networks.

- Cross-specialty investigator engagement
- Centralized pathology and imaging coordination
- Organ-specific endpoint delivery across global sites

For multisystem diseases, consistent endpoint execution is essential. In systemic sclerosis, key assessments may include pulmonary function (FVC), skin fibrosis (mRSS), and high-resolution CT imaging (HRCT). Our established network of specialty vendors and centralized partners ensures high-quality, consistent data across every organ domain.

Ensuring data quality with scientific surveillance

In complex indications such as systemic sclerosis, our scientific surveillance model combines subject-level clinical insight with advanced statistical monitoring to strengthen trial oversight.

By applying clinically relevant rules and detecting systematic patterns rather than random variation, we support earlier, more meaningful intervention while maintaining data integrity. Cross-functional review, including pharmacovigilance expertise, ensures robust efficacy and safety oversight across the trial life cycle. We also provide real-time eligibility and efficacy data review to manage complex inclusion criteria.

Through our partnership and experience working with the leading clinical trial endpoint technology companies operating in this multi-disciplinary space, we enhance evidence generation with specialized data collection, analysis, and trial support solutions. Spanning eCOA, medical imaging, cardiac safety, respiratory, and a wide range of digital endpoint technologies, these capabilities are critical to advancing innovation in rare immunology indications.

Bring rare immunology therapies to patients sooner with our integrated scientific and clinical trial expertise

ppd.com/therapeutic-expertise/immunology/

Advanced cell therapy in rare autoimmunity

Cell therapy trials demand a clinical development partner with specialized expertise and flawless execution. Our teams manage complex scheduling and logistics, oversee vendors and laboratories, and provide end-to-end visibility to reduce risk and variability. We also deliver patient-centric support—including education, travel and reimbursement assistance, and concierge-style coordination—to enhance enrollment, retention, and the overall study experience, while maintaining rigorous quality and compliance.

That expertise is backed by recent experience in autoimmune cell therapy. Over the past three years, we have supported 16 cell therapy studies across autoimmune indications, including systemic lupus erythematosus, lupus nephritis, ANCA-associated vasculitis, systemic sclerosis, Sjogren's syndrome, and idiopathic inflammatory myositis.

Our delivery model combines oncology cell therapy experience with deep immunology expertise, advancing innovative therapies into autoimmune disease development through:

- A customized cell therapy feasibility approach
- In-house site support to accelerate site activation and patient recruitment

An industry leader in cell therapy clinical research, we leverage our extensive experience and industry-leading capabilities to address these challenges and support your cell therapy clinical trials

Why choose us?

An experienced team that will keep your rare immunology development on track

We provide industry-leading services

As a leading global provider of a full suite of innovative CDMO (contract development manufacturing organization) and clinical research services, as well as a partnership with Trialmed, we provide the tools needed for advancing research in rare immunology indications.

With integrated drug development capabilities, flexible site and patient solutions, and deep rare immunology expertise, we enable sponsors to accelerate complex programs, enhance execution, and generate high-quality evidence for underserved patient populations.

trialmed

Accelerator™ Drug Development | 360° CDMO and CRO solutions

Bring rare immunology therapies to patients sooner with our integrated scientific and clinical trial expertise

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