JUNE 2026

SAVOIR & COMPRENDRE
AVANCÉES DE LA RECHERCHE

Advances in research 2026

Inflammatory myopathies (myositis)

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Explore!

A selection of research news stories from the past year (ongoing observational studies and clinical trials, scientific publications, etc.).
A comprehensive overview created for the AFM-Téléthon General Meeting 2026.

AFMTÉLÉTHON
INNOVER POUR GUÉRIR

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Inflammatory myopathies — Myositis

Myositis is the name of a group of diseases called inflammatory myopathies which are linked to inflammation caused by autoimmunity. They result from immune system dysfunction which leads to the production of pathological antibodies (autoantibodies), causing tissue damage (muscle, skin, lungs, heart, etc.).

Common symptoms

- **Muscle symptoms** may include muscle weakness (fatigue, lack of strength), muscle pain, swallowing difficulties and voice changes.
- **Non-muscle symptoms** may include rashes, temporary blood flow problems (Raynaud's phenomenon), joint pain, shortness of breath and significant fatigue.
- Symptoms **vary greatly** from patient to patient, and from one type of myositis to another.

Five main types

Dermatomyositis	• Onset in childhood or adulthood • Muscle inflammation and/or rashes caused by blood vessel damage • Activation of the type 1 interferon pathway with complement deposition.
Inclusion body myositis	• Onset after the age of 30 • First muscles affected are those of the thighs and fingers • Toxic protein aggregates ("inclusions") in muscle fibres • Conventional myositis treatments ineffective.
Immune-mediated necrotising myopathy	• Onset can occur at any age, usually around the age of 40 • Sometimes caused by cholesterol-lowering drugs (statins) • Significant muscle necrosis seen on biopsies with little or no inflammation.
Antisynthetase syndrome	• Onset can occur at any age, usually around the age of 30 • Its three main features are muscle inflammation, lung disease and joint inflammation (the "classic triad) • Specific autoantibodies (anti-Jo1, anti-PL7, anti-PL12, etc.).
Overlap myositis	• Onset in childhood or adulthood • Involves muscle symptoms combined with those of other systems (skin, lungs, joints, etc.) • Autoantibodies linked to other diseases (anti-PM/Scl, anti-Ku, anti-RNP) such as scleroderma.

(The existence of polymyositis is debated, with most patients being reclassified into one of the five categories above)

In numbers

≈ **1** person in every **7,000** has an inflammatory myopathy

Over 1,400 scientific articles published between May 2025 and May 2026 *(PubMed)*

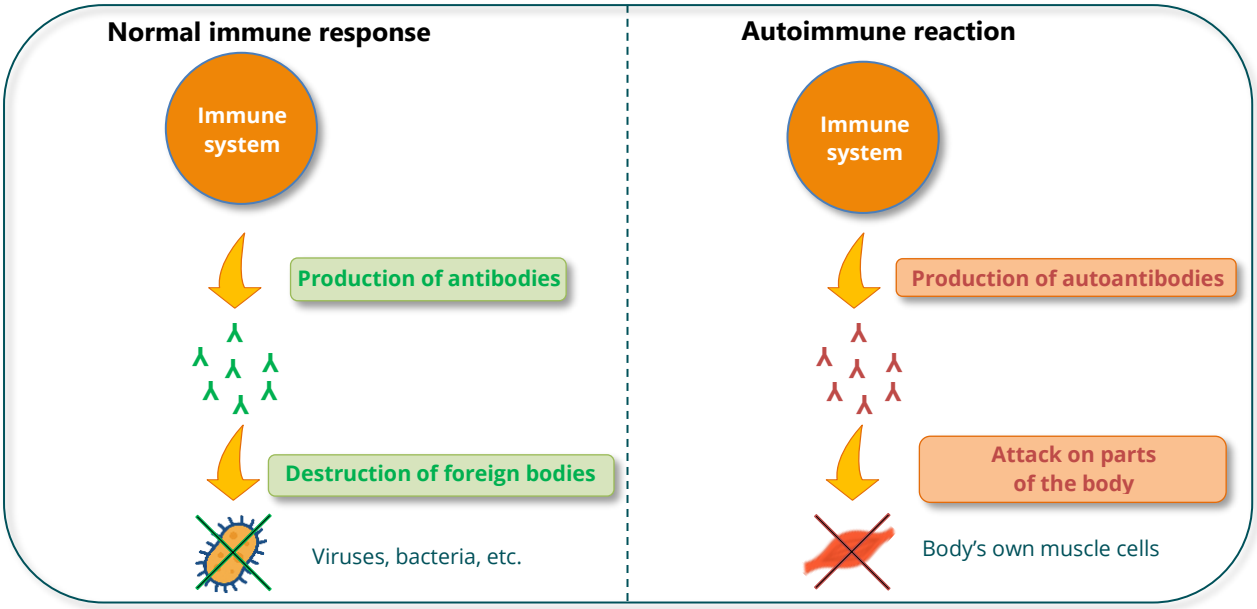
192 clinical trials and observational studies including **36** in France *(ClinicalTrials.gov 28/05/2026)*

What causes myositis?

Genetic predisposition
+ Environmental factors
(medications, UV rays, smoking, viral infections, etc.)

→ **Immune system dysfunction**

Autoimmune reaction with production of
- **myositis-specific autoantibodies,**
- or **myositis-associated autoantibodies**, which can also be present in other autoimmune diseases.



Some myositis-specific autoantibodies

Dermatomyositis	**Immune-mediated necrotising myopathy**	**Antisynthetase syndrome**
Anti-Mi2, anti-Tif1-γ, anti-NXP2, anti-MDA5, anti-SAE	Anti-SRP, anti-HMGCR	Anti-Jo1, anti-PL7, anti-PL12, anti-OJ

Current treatments (excluding inclusion body myositis)

Corticosteroids, immunosuppressants and immunomodulators

- Reduce immune system activity.
- Prednisone, methotrexate, azathioprine, tacrolimus.

Targeted therapies

- Find and attack target molecules (cells, proteins) involved in the immune response.
- Rituximab (MabThera®).

Intravenous immunoglobulin therapy

- Uses antibodies taken from healthy donors that are capable of modulating immune

Plasmapheresis

- Uses a machine that filters blood and purifies it from substances such as autoantibodies.

For more information on inflammatory myopathies, please visit:
www.afm-telethon.fr/fr/fiches-maladies/myosites-myopathies-inflammatoires [page in French]

3 highlights from the past 12 months

1 Progress in childhood-onset myositis

What's the latest?

Several teams of doctors (including one French team) have recently published positive results regarding the use of two drugs (dazukibart and anifrolumab) in children with dermatomyositis that is resistant to other drugs. On top of this, 12 clinical trials around the world involving children and adolescents with myositis are currently taking place or preparing to start. Five of these trials involve CAR-T cell therapy - a combination of cell therapy and gene therapy.

Why is this significant?

Because clinical trials involving children and adolescents in any disease are rare. CAR-T cell therapy (which is relatively new in myositis) was initially only available to adults.

2 Disappointment for ulviprubart in inclusion body myositis

What were the results?

The "Muscle" trial evaluated ulviprubart in 272 patients around the world, including in one investigator site in Paris. Preliminary results shared during the Global Conference on Myositis (GCOM) in Portugal at the end of March 2026 were rather negative (the drug candidate did not achieve the trial's endpoints). However, the participants with mild to moderate inclusion body myositis did experience a slowing in disease progression.

And now?

The pharmaceutical company which is developing ulviprubart is planning to continue its development in a subgroup of patients (those in the early stages of the disease). *Abcuro. 2026(1)* *Abcuro. 2026(2)*

3 Transcriptomics is gaining popularity

What is it?

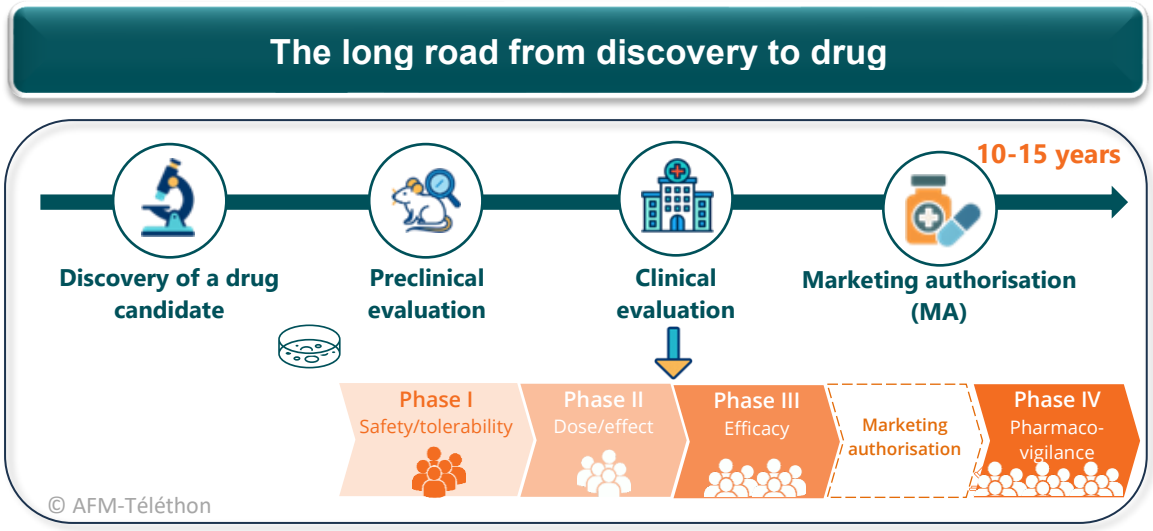
Transcriptomics consists of analysing the transcriptome, that is, the set of RNA molecules or "transcripts" (which are copies of genes) produced by a cell at a given moment. Not all genes are active at all times - their activity varies according to the state of the cell (onset of disease, response to treatment, etc.). Analysing these RNA transcripts makes it possible to understand which genes are active a given moment and how the cell is reacting.

And in myositis?

Transcriptomics is useful in non-genetic diseases (such as inflammatory myopathies) where the expression of certain genes leads to, for example, the production of molecules that promote or even reduce inflammation.

Over the past year, around 30 publications have reported results from research based on transcriptomics used to study different types of myositis with the aim of better understanding the mechanisms of these diseases and the effects of certain treatments.

Messenger RNA (mRNA) [page in French]



From laboratory to pharmacy - a heavily regulated journey

✓ **Research and discovery.** Everything begins in a laboratory when researchers identify a new drug candidate (a promising molecule, a new therapeutic approach like gene therapy).

✓ **Preclinical studies.** This drug candidate is then tested in the laboratory in cell models (*in vitro*) and animal models (*in vivo*) of the disease in order to study its mode of action, pharmacokinetics and pharmacodynamics, efficacy, and toxicity. This step also makes it possible to estimate a first dose that can be safely used in humans.

✓ **Clinical trials.** If the results are conclusive, the drug candidate is then evaluated in patients in clinical trials over **several phases** in order to check whether it is well tolerated (**phase I**), what the optimal dose is (**phase II**) and whether it is effective (**phase III**).
In rare diseases, certain phases can be grouped together (phase I/II, phase II/III, etc.).

✓ **Marketing authorisation (MA).** Once these steps have been validated, the European Medicines Agency (EMA) grants marketing authorisation (MA). Then, each country determines its own marketing conditions (price, reimbursement) which can take several months before the drug is made available in the country.
Once the drug has received marketing authorisation, it remains under close surveillance (**phase IV - pharmacovigilance**) to monitor its effects in "real life" over time.

✓ In France, there are access schemes for drugs that do not yet have MA (**compassionate use** and **early access**).

✓ With the exception of intravenous immunoglobulin therapy in dermatomyositis, none of the immunosuppressants or immunomodulators commonly used to treat inflammatory myopathies have MA for these diseases. They are prescribed based on study results, expert recommendations and clinical experiences from specialist rare disease centres.
These treatments are therefore routinely prescribed **off-label**, outside of compassionate use and early access schemes, under the supervision of the doctor prescribing the treatment, usually at a specialist centre for the disease in question.

Les essais cliniques en pratique [Clinical trials explained]
"Essais cliniques et Maladies neuromusculaires" ["Clinical trials and neuromuscular diseases"] reference documents

The majority of clinical trials described in this document are accompanied by an "NCT" number (written as "NCT0XXXXXXX") - an identification number assigned to each clinical trial on ClinicalTrials.gov, the most comprehensive clinical trials database in the world.
Clicking on this number in the text will open the corresponding trial's description page.

Improving treatments

What's the problem?

Thanks to currently available treatments, some patients will only ever have one flare-up of their disease in their lifetime, however:

- for others, the disease becomes chronic, which can lead to long-term complications in the muscles, lungs, etc.,
- drugs can cause adverse events requiring them to be discontinued, and some forms of myositis may be resistant to treatments ("refractory"),
- current drugs are not effective in inclusion body myositis.

Clinical trials make it possible to evaluate new ways of treating myositis.

Which treatments are currently in trials in myositis in France?

Treatment	Approach	Recruitment
Stem cells (ADSVF-in-IBM trial) ➥ Inclusion body myositis	Cell therapy	Not recruiting
YTB323 CAR-T cells ➥ Several types of myositis	Gene and cell therapy	Recruiting
CC-97540 CAR-T cells (Breakfree-1 trial) ➥ Several types of myositis	Gene and cell therapy	Recruiting
BMS-986515 CAR-T cells NEW ➥ Several types of myositis	Gene and cell therapy	Recruiting
FT819 CAR-T cells NEW ➥ Several types of myositis	Gene and cell therapy	Recruiting
AlloNK® (or AB-101) cells NEW ➥ Several types of myositis	Cell therapy	Recruiting
Anifrolumab (JASMINE trial) ➥ Several types of myositis	Pharmacology	Recruiting
AZD5492 (TITAN trial) ➥ Dermatomyositis and polymyositis	Pharmacology	Recruiting
Baricitinib (BIRD trial) ➥ Dermatomyositis	Pharmacology	Recruiting
Dazukibart (or PF 06823859) ➥ Dermatomyositis and polymyositis	Pharmacology	Recruiting
Efgartigimod (ALKIVIA trial) ➥ Several types of myositis	Pharmacology	Not recruiting
Efgartigimod extension (ALKIVIA+ trial) ➥ Several types of myositis	Pharmacology	Recruiting
GLPG3667 (GALARISSO trial) ➥ Dermatomyositis	Pharmacology	Not recruiting
Nipocalimab (SPIREA trial) ➥ Several types of myositis	Pharmacology	Not recruiting
RAY121 (RAINBOW trial) ➥ Several types of myositis	Pharmacology	Recruiting
RAY121 (RAINBOW-LTE trial) NEW ➥ Several types of myositis	Pharmacology	Recruiting
Ruxolitinib (BIGTIM trial) ➥ Inclusion body myositis	Pharmacology	Recruiting
Ulviprubart (ABC008) extension ➥ Inclusion body myositis	Pharmacology	Not recruiting
Sodium thiosulfate (ITS-PILOT trial) ➥ Dermatomyositis	Pharmacology	Recruiting

Innovative non-pharmacological treatments in trials

From one approach to another

The pharmacological approach uses drugs (chemically-derived drugs, biologics such as antibodies) that interact with specific targets and correct or modify a given function.

Gene and cell therapies are non-pharmacological approaches that consist of correcting or replacing faulty genes or cells in the body.

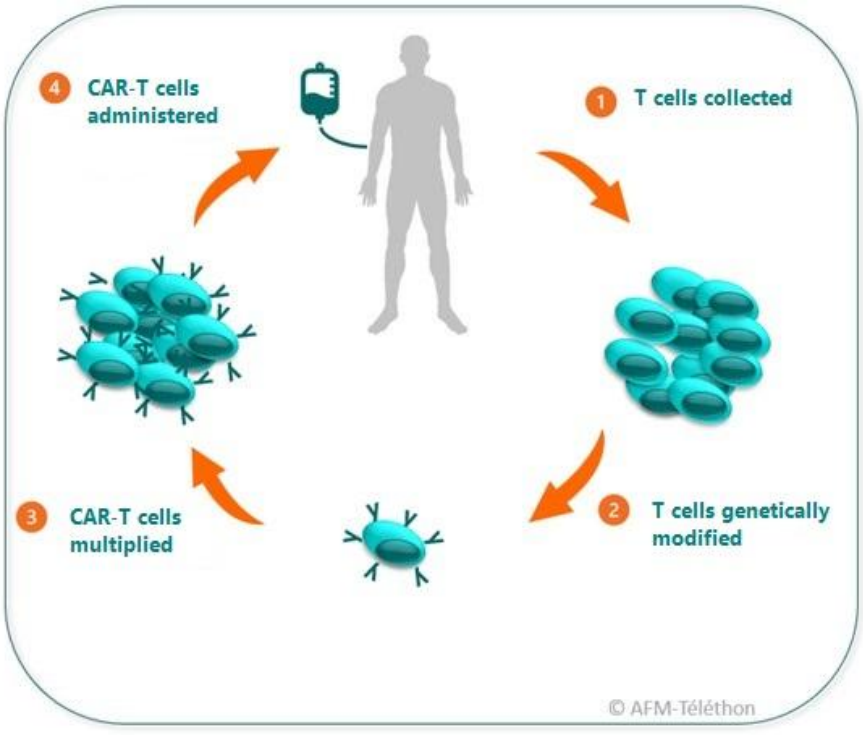
CAR-T cell therapy

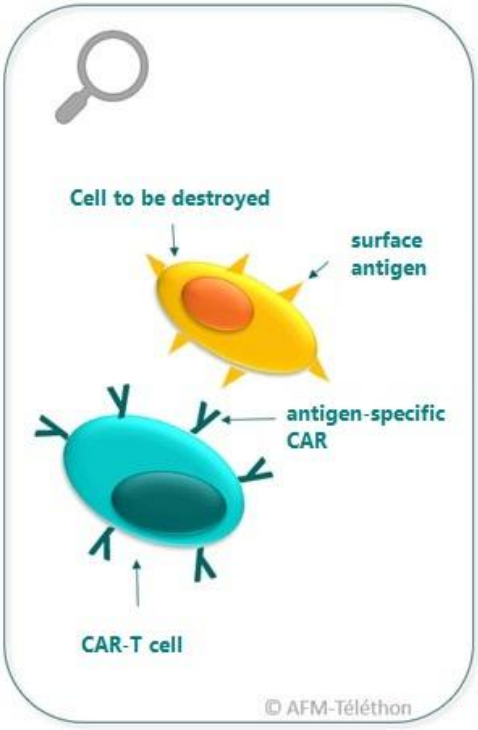
⇨ How does it work?

CAR-T cell therapy originates from cancer research and is a combination of cell therapy and gene therapy. CAR-T cells are T cells (a type of white blood cell) that are able to destroy a given type of cell. They are:

- usually collected from the patient to be treated ("autologous" T cells),
- genetically modified in a laboratory to become CAR-T cells which are capable of recognising specific antigens that are present on the surface of the cells to be destroyed (CAR stands for chimeric antigen receptor),
- injected into the patient, usually after very powerful immunosuppressive treatment has been administered to weaken the immune system and remove any natural T cells from the body.

In autoimmune diseases, the aim is to destroy immune cells that produce pathogenic autoantibodies (B cells).





AFM-Téléthon and CAR-T cell research

With the support of AFM-Téléthon, several teams of researchers in France worked on using CAR-T cells to treat various neuromuscular diseases, including:

- Prof. Olivier Boyer's team (CHU Rouen [Rouen University Hospital]) on developing CAR-T cells in immune-mediated necrotising myopathy;
- Inès Barthélemy's team (École Nationale Vétérinaire d'Alfort [National Veterinary School of Alfort]) on creating CAR-T cells to tackle fibrosis in Duchenne muscular dystrophy.

⇨ Who is it for?

Patients with a severe form of myositis that is resistant to conventional therapies (inclusion body myositis excluded).

⇨ What's new?

Trends

- A very significant increase in the number of CAR-T cell clinical trials in various different types of myositis in adults, but also in children and adolescents.
- The rise of "ready-to-use" alternatives to T cells from patients, such as T cells from healthy donors that have been modified in a laboratory (allogeneic CAR-T cells) to reduce the risk of being rejected by patients, and stem cells. These options save time, lower costs and make it possible to treat more patients.

Results

Promising results have been observed in antisynthetase syndrome, dermatomyositis (including juvenile dermatomyositis) and immune-mediated necrotising myopathy, with improvements in symptoms (up to remission), however, questions remain regarding the extent of the benefits and the duration of efficacy (initial treatment failures and relapses reported), long-term side effects, etc.

Jouret M et al. 2025 *Müller F et al. 2026* *Cheng J et al. 2026* *Müller F et al. 2025*

Good to know

- There are currently 45 CAR-T cell trials in myositis underway or preparing to start (vs three in 2023 and 29 last year) around the world, including a total of over 2,000 participants with dermatomyositis (four trials) or various other autoimmune diseases, including inflammatory myopathies.
- The various types of CAR-T cells currently in trials all target B cells and are distinguished by the surface antigen that they are directed against (CD19, BCMA, CD20, etc.).

⇨ What else has been happening?

- A team produced CAR-T cells *in vivo* in mice, that is, the T cells were modified in the mice themselves instead of in a laboratory (*ex vivo*). Two trials taking place in China (NCT07413341 and NCT07339540) are evaluating *in vivo* CAR-T cells in various different autoimmune diseases, including myositis. *Nyberg WA et al. 2026*

Learn more [French]

- Alternatives to CAR-T cells called bispecific antibodies (also known as T cell engagers) bind to both T cells and B cells, causing the T cells to the kill the B cells. Examples of these include blinatumomab, teclistamab and glofitamab. Publications have reported their success, including in severe forms of antisynthetase syndrome.

Düsing C et al. 2026 *Wei C et al. 2025* *Phithak E et al. 2026*

⇨ CAR-T cell trials in myositis in France

Name	Trials	Stage	Key points
CC-97540 (BMS-986353)	NCT05869955 Breakfree-1 trial	Phase I	• CD19-targeted CAR-T cells • derived from patients
Rapcabtagene autoleucel (or **YTB323)**	NCT06665256	Phase II	• CD19-targeted CAR-T cells • derived from patients
BMS-986515 NEW	NCT07115745	Phase I	• CD19-targeted CAR-T cells • derived from healthy donors (allogeneic)
FT819 NEW	NCT06308978	Phase I	• CD19-targeted CAR-T cells • derived from stem cells • adolescents and adults

CC-97540 cells (Breakfree-1)

- Trial involving patients with severe, refractory forms of various autoimmune diseases, including dermatomyositis, polymyositis, immune-mediated necrotising myopathy and antisynthetase syndrome.
- Sites in France (Bordeaux, Lille, Montpellier, Nice, Paris, Rennes and Strasbourg).

Breakfree-1 trial (phase I)			NCT05869955
France and abroad	270 (over 18 years old)	Sept 2023 – Aug 2028 2 years of follow-up	Intravenous

Preliminary results in myositis

According to a presentation made during the ACR Convergence meeting at the end of 2025 in Chicago, 10 out of 11 myositis patients (91%) experienced moderate to major improvements. At their most recent follow-up visits (ranging from five to 12 months after treatment depending on the patient) they no longer required immunosuppressants.

Aggarwal R et al. 2025

YTB323 cells (rapcabtagene autoleucel)

- Trial involving patients with severe, refractory forms of myositis (inclusion body myositis excluded).
- Five investigator sites in France (Brest, Lille, Lyon, Paris and Rennes).

YTB323 (rapcabtagene autoleucel) trial (phase II)			NCT06665256
France and abroad	123 (18 to 75 years old)	Dec 2024 – July 2030 1 year of follow-up	Intravenous

BMS-986515 cells

- Trial involving patients with severe, refractory forms of various autoimmune diseases, including myositis (inclusion body myositis excluded).
- Two investigator sites in France (Lille and Strasbourg).

NEW

BMS-986515 trial (phase I)			NCT07115745
France and abroad	125 (12 to 70 years old)	Sept 2025 – Aug 2030 2 years of follow-up	Intravenous

FT819 cells

NEW

- Trial involving patients with severe, refractory forms of various autoimmune diseases, including myositis (inclusion body myositis excluded).
- One investigator site in France (Hôpital Pitié-Salpêtrière in Paris).

FT819 trial (phase I)			NCT06308978
France and abroad	244 (over 18 years old)	March 2024 – Sept 2042 2 years of follow-up	Intravenous

NK cell-based cell therapy

⇨ How does it work?

The patient receives:

- immunosuppressive chemotherapy,
- rituximab which targets B cells (the cells that produce autoantibodies),
- AlloNK® (AB-101) cells to enhance the effect of rituximab on B cells.

AlloNK® cells are derived from umbilical cord blood. They are natural killer (NK) cells - immune cells capable of selectively destroying specific diseased cells.

⇨ An ongoing trial

- Trial involving patients with severe, refractory forms of various autoimmune diseases, including myositis (inclusion body myositis excluded).
- Three investigator sites in France (Marseille, Montpellier and Toulouse).

NEW

AlloNK® trial (phase II) NCT06991114

France and abroad	90 (over 18 years old)	July 2025 – Jan 2029 2 years of follow-up	Intravenous

Recent results in blood cancer

At the start of 2026, promising results (in terms of efficacy, safety and tolerability) from a clinical trial that combined AlloNK® and rituximab in relapsed/refractory non-Hodgkin lymphoma (a type of blood cancer caused by the uncontrolled proliferation of B cells) were published.
Farooq U et al. 2026

Umbilical cord blood-derived stem cells

⇨ How do they work?

- Stem cells have the ability to differentiate into new cells, including immune cells, that do not attack components of the body (non self-reactive).
- This type of treatment has already been approved for very severe forms of certain autoimmune diseases, including dermatomyositis and polymyositis.
- Umbilical cord blood-derived stem cells (placenta donation) are more immature and therefore will potentially be better tolerated by their recipient than stem cells from other sources (bone marrow donation, etc.).

Learn more [French]

⇨ A dermatomyositis and polymyositis trial

NEW

In the United States, a trial (NCT07160205) is evaluating the efficacy, safety and tolerability of three infusions of umbilical cord blood-derived stem cells in 40 adults with dermatomyositis or polymyositis against a placebo (a substance that contains no active ingredient).

Mitochondria

⇨ How do they work?

Mitochondria, the powerhouses of cells, do not work properly in myositis. PN-101 is a treatment that uses mitochondria extracted from umbilical cord blood-derived stem cells.

⇨ An upcoming trial

NEW

A new phase II trial (NCT07122648) in South Korea will be recruiting 36 patients with a refractory form of dermatomyositis or polymyositis. They will receive either PN-101 or a placebo.

In inclusion body myositis

What's the problem?

- Diagnosis is often delayed (five to eight years after the onset of symptoms) as the disease progresses slowly and develops relatively late in life, meaning the muscle weakness may be attributed to another cause.
- No immune-targeted treatments used in other types of myositis have so far proven to be beneficial in inclusion body myositis, however, several different treatment options are currently being explored.

Good to know
In France, **over 800** people are monitored by a specialist centre for inclusion body myositis according to data collected by the BNDMR (Banque Nationale de Données Maladies Rares [French National Rare Disease Registry]).
BNDMR national report. February 2026 [report available in English]

What treatments are currently in trials?

Name	Trials	In France	Stage	Key points
Cell therapy	NCT05032131	Yes	Phase I	• not recruiting • supported by AFM-Téléthon
Ruxolitinib (Jakavi®)	NCT06536166 BIGTIM trial	Yes	Phase II	• recruiting • supported by AFM-Téléthon
Ulviprubart (ABC008)	NCT05721573 NCT06450886	Yes	Phase II/III	• not recruiting • preliminary results released
Pozelimab + Cemdisiran	NCT06479863	No	Phase I	• United States only
Rapamycin (sirolimus)	NCT04789070	No	Phase III	• sites in multiple countries • immunosuppressant used to prevent organ transplant rejections • prior pilot study (France, 2015-2018) supported by AFM-Téléthon

Cell therapy

⇨ How does it work?

Learn more [French]

- By transplanting cells (which are usually derived from stem cells) in order to restore the function of an organ or a type of tissue such as muscle.

⇨ The ADSVF-in-IBM trial in France

- Trial evaluating a type of stem cell (mesenchymal stem cells) which are taken from the patient to be treated, then modified in a laboratory before being injecting back into their finger flexor muscles (forearm).
- One investigator site (Hôpital Pitié-Salpêtrière in Paris).
- Recruitment complete, analysis of results expected late 2026/early 2027.
- The investigators have not observed any toxicity during this stage.

ADSVF-in-IBM trial (phase I) NCT05032131

France	32 (45 to 80 years old)	Started in Feb 2023 6 months of follow-up	Intramuscular

Cell therapy in the lab
A French team tested the intramuscular injection of stem cells derived from fat tissue in mice with a form of spontaneous myositis, yielding positive results (slowing in disease progression, improvements in motor functions such as gait, reduction in muscle atrophy).
Pileyre B et al. 2025

Ruxolitinib (Jakavi®)

⇨ How does it work?

By inhibiting Janus kinases (JAK), enzymes whose activation occurs in inflammation pathways involving interferon-gamma (IFN-γ). When administered in mice, IFN-γ causes premature muscle aging similar to that seen in inclusion body myositis, and delays muscle repair. Ruxolitinib counteracts these effects.

⇨ The BIGTIM trial

- A placebo-controlled trial evaluating the efficacy of ruxolitinib (Jakavi®).
- Recruiting in France only in specialist centres in the FILNEMUS rare diseases healthcare network [page in French].
- Participants must have weakness in the quadriceps (thigh muscles) or finger flexors, a muscle biopsy showing signs of inclusion body myositis, and be able to walk for six minutes without help from another person.

Learn more [French]

BIGTIM trial (phase II) NCT06536166

France	80 (over 45 years old)	May 2025 – Sept 2028 15 months of follow-up	Oral (tablets)

Ulviprubart (ABC008)

⇨ How does it work?

By selectively depleting cytotoxic KLRG1+ T cells which:
- are found in the muscles and blood of individuals with inclusion body myositis,
- gradually destroy muscle fibres over time,
- are resistant to corticosteroids and immunosuppressants.

⇨ What's new?

- Preliminary results from the MUSCLE trial (NCT05721573) showed that ulviprubart was well tolerated but did not achieve the trial's endpoints.
- The participants with a mild to moderate form of the disease did however see a slower decline in muscle strength, suggesting that ulviprubart could continue to be developed specifically in this population.

Abcuro. 2026(1) *Abcuro. 2026(2)*

Pozelimab and cemdisiran (combination therapy)

⇨ How do they work?

Pozelimab (Veopoz®) is a monoclonal antibody directed against complement component 5 (C5). Cemdisiran is a small interfering RNA molecule that also targets C5.

Put simply

- **Complement** is an immune response mediator which circulates in the blood. It is made up of several proteins, including C5.
- A small interfering RNA (**siRNA**) molecule binds specifically to a messenger RNA (mRNA) molecule, to which it is complementary. In doing so, it prevents the mRNA molecule from being translated into a protein.

⇨ Are there clinical trials?

In the United States, an expert centre is conducting a pilot study (NCT06479863) until 2027 to evaluate pozelimab/cemdisiran combination therapy in 10 adults with inclusion body myositis.

Rapamycin (sirolimus)

⇨ What is it?

Learn more [French]

An immunosuppressant used to prevent rejection after a kidney transplant. It is being evaluated in a trial (NCT04789070) taking place in several countries, with 140 participants already recruited.

Mixed or very preliminary results

⇨ A pilot study of pioglitazone

- Pioglitazone, which is used as an antidiabetic drug in some countries, reportedly improves the function of muscle cell mitochondria which do not work properly in inclusion body myositis. In the United States, 13 inclusion body myositis patients took one pioglitazone tablet a day for seven months as part of a trial (NCT03440034).
- The primary endpoint of the trial (change in *PPARGC1A* gene expression and related metabolic pathways in muscle) was not achieved, however, the investigators did notice a trend towards an increase in *PPARGC1A* expression, as well as a positive effect on certain functional test scores (IBM-FRS, Modified Timed Up and Go) in one subgroup of participants. *Adler BL et al. 2026*

⇨ A gene therapy product in the lab

- An American company developed a gene therapy product (AVGN7.2) for non-genetic diseases (such as inclusion body myositis) and genetic diseases (such as Duchenne muscular dystrophy) with the aim of halting muscle degeneration and increasing muscle mass by blocking myostatin.
- In order to achieve this, a viral vector (AAV6) is used to transport the *SMAD7* gene which codes for a protein that blocks the actions of myostatin.
- A preclinical study in mice showed that AVGN7.2 was well tolerated.
- The viral vector was detected in the animals' livers and muscles, and the SMAD7 protein was overexpressed in their muscles. *Herring KS et al. 2026*

Learn more [French]

A large-scale trial of bimagrumab (a monoclonal antibody that blocks myostatin) previously conducted in over 200 inclusion body myositis patients ended by failure.

Observational studies and databases

⇨ A natural history study

- In California, 150 adults with inclusion body myositis are participating in the INSPIRE-IBM study (NCT05046821) until March 2027.
- Its main objective is to evaluate whether NT5c1A antibodies influence disease progression.

⇨ Sensors for monitoring muscle function

- A study (NCT06153108) conducted in the United States involving 20 patients is aiming to validate the use of wearable sensors for monitoring upper limb function during activities of daily living.
- This type of monitoring could provide a more accurate reflection of reality than a test performed during a consultation, and could therefore be used in clinical trials evaluating new treatments.

⇨ A dedicated registry

Yale University (United States) has been developing the Inclusion Body Myositis Disease Registry (IBMR) since 2012. Patients who wish to join the registry can fill in an online questionnaire on the registry's website. *Registry's website*

In dermatomyositis and polymyositis

What's the problem?

Various different drugs are available to treat dermatomyositis and polymyositis, however:
▪ they do not always help to improve the manifestations of dermatomyositis,
▪ they may also be contraindicated or cause significant side effects. This is referred to as a "refractory" form of the disease.

Good to know
In France, **over 2,000** people are monitored by a specialist centre for dermatomyositis, and **675** for polymyositis, according to data collected by the BNDMR.
BNDMR national report. February 2026 [report available in English]

What treatments are currently in trials?

▪ Almost 50 clinical trials around the world that are currently underway or preparing to launch are evaluating drug candidates specifically in dermatomyositis and polymyositis.
▪ In addition, there are trials that are also recruiting patients with other types of myositis (For more information see "In several different types of myositis" and "CAR-T cell therapy").

Three Janus kinase inhibitors

Name	Trials	In France	Stage	Key points
Baricitinib	NCT04972760 BIRD trial	Yes	III	• JAK1 and JAK2 inhibitor • oral administration • recruiting
GLPG3667	NCT05695950 GALARISSO trial	Yes	II	• TYK2 inhibitor • oral administration • not recruiting
Ruxolitinib	NCT06857240	No	II	• JAK1 and JAK2 inhibitor • topical application (cream) • recruiting

⇨ How do they work?

▪ By inhibiting JAK, enzymes necessary for the activation of certain signalling pathways involving interferons which can result in chronic inflammation.
▪ Dermatomyositis is thought to be an interferonopathy.
▪ There are four enzymes in the JAK family: JAK1, JAK2, JAK3 and tyrosine kinase 2 (TYK2). JAK inhibitors (drugs that inhibit one or several JAK) already have MA for the treatment of other autoimmune diseases.

The pioneering Institut de Myologie
In 2018, a team from the Institut de Myologie [Institute of Myology] in Paris, supported by AFM-Téléthon, helped demonstrate the benefits of ruxolitinib (a JAK inhibitor) in dermatomyositis resistant to conventional therapies. Since then, several clinical trials have evaluated different JAK inhibitors in refractory inflammatory myopathies.

⇨ What's new?

Data on JAK inhibitors published in recent months:
▪ results from the VALOR trial (NCT05437263) conducted in several countries (not in France) in 225 adults with refractory dermatomyositis showed that brepocitinib use (30 mg) led to significant benefits compared to the placebo,

▪ a study in China demonstrated the safety and efficacy (on skin manifestations, calcinosis, corticosteroid doses) of baricitinib in 27 children with a severe form of dermatomyositis who were monitored for three years,
▪ a study in France demonstrated the efficacy of three JAK inhibitors (baricitinib, ruxolitinib and tofacitinib) in 32 out of 39 patients with new-onset or refractory juvenile dermatomyositis.
Vleugels R et al. 2026 *Wang Z et al. 2025* *Bader-Meunier B et al. 2025*

⇨ Are there any clinical trials currently taking place?

Baricitinib
▪ Placebo-controlled trial in adults with active relapsing or treatment-naive dermatomyositis.
▪ One investigator site (Hôpital Pitié-Salpêtrière in Paris).

Placebo-controlled BIRD trial (phase III) NCT04972760

France	62 (18 to 64 years old)	Started in Aug 2022 6 months of follow-up	Oral (tablets)

GLPG3667
▪ Placebo-controlled trial in adults with refractory dermatomyositis.
▪ Three investigator sites in France (Nice, Paris and Strasbourg).

Placebo-controlled GALARISSO trial NCT05695950

France and abroad	40 (18 to 75 years old)	Started in Feb 2023 1 year of follow-up	Oral (capsules)

Topical ruxolitinib
In the United States, a phase II clinical trial (NCT06857240) is exploring the efficacy, safety and tolerability of applying ruxolitinib cream (Opzelura®) to the skin manifestations of 15 adults with refractory dermatomyositis for three months.

Two anti-complement antibodies

Why target complement?
▪ Found in the blood, complement comprises several proteins (called components) involved in immune reactions.
▪ When activated, its components 5 to 9 form the membrane attack complex (MAC) that binds to the surface of microorganisms during infections.
In dermatomyositis, the MAC binds to cells that line the inside of small blood vessels (in skin, muscle, etc.), causing the damage that leads to the manifestations of the disease.
▪ Treatment with anti-complement antibodies can block complement activation and prevent the formation of the MAC.

⇨ What's new?
Results for eculizumab (Soliris®):
▪ this drug, which already has MA for the treatment of other autoimmune diseases, binds specifically to complement component 5 (C5),
▪ in late 2025, a French team reported the success of eculizumab treatment in two adults with relapsing anti-NXP2 dermatomyositis. *Combemale L et al. 2025*

⇨ How do they work and are there clinical trials?

Empasiprubart

- Empasiprubart (or ARGX-117) is an intravenously-administered antibody that targets and inhibits complement component 2 (C2).
- The phase II, placebo-controlled EMPACIFIC trial (NCT06284954) is evaluating it in 42 adults with dermatomyositis in the United States and other countries (not in France).

Ruxoprubart

- Ruxoprubart (or NM 8074) inhibits factor Bb which blocks the "alternative" complement pathway. It is administered via intravenous infusion.
- A phase II clinical trial (NCT06887738) involving 15 adults with dermatomyositis is expected to start by the end of 2026. The list of participating countries has not been published yet.

Two anti-interferon antibodies

⇨ How do they work?

- By blocking type I interferons (inflammatory mediators that help regulate immune reactions).
- Dermatomyositis is thought to be an interferonopathy, meaning that it involves an overexpression of type I interferon-dependent genes.

⇨ What's new?

Positive results have been published in recent months concerning two anti-type I interferon antibodies:

- anifrolumab - effective in adults and children with refractory dermatomyositis, particularly on skin manifestations,
- dazukibart - effective in children with dermatomyositis, particularly after the failure of JAK inhibitor treatment.

Rahbek SJ et al. 2025 *Barrutia-Etxebarria A et al. 2025* *Windschall D et al. 2026*
Fournier B et al. 2025 *Guirguis J et al. 2025*

⇨ Are there any clinical trials currently taking place?

Anifrolumab (Saphnelo®)

- Trial evaluating anifrolumab injected once a week, initially in a placebo-controlled format (participants will receive anifrolumab or placebo for one year), then open-label (all participants will receive anifrolumab for one year), in patients with moderate to severe dermatomyositis or polymyositis.
- Eleven investigator sites in France where recruitment is currently taking place.

Placebo-controlled JASMINE trial (phase III)			NCT06455449
France and abroad	240 (18 to 75 years old)	June 2024 – Aug 2028 1 year of follow-up	Subcutaneous

Dazukibart (or PF 06823859)

- Dazukibart is an antibody targeting interferon-beta 1. It is being evaluated in a placebo-controlled trial in adults with dermatomyositis or polymyositis.
- Two investigator sites in France (Reims and Strasbourg).

Placebo-controlled dazukibart trial (phase III) NCT05895786

France and abroad

318 (over 18 years old)

May 2023 – July 2026
1 year of follow-up

Intravenous

Participants who complete this initial trial will be asked whether they want to participate in an open-label extension (NCT06698796) lasting one additional year.

Two bispecific antibodies

⇨ How do they work?

- By mobilising immune cells (T cells for AZD5492, myeloid cells for DR-0201) so that they attack B cells which are recognised by their CD20 surface receptors.
- B cells become plasma cells which produce antibodies. These include the autoantibodies that are involved in the development of autoimmune diseases.

⇨ Are there clinical trials?

AZD5492

- Open-label trial (no placebo) in various different diseases, including refractory dermatomyositis.
- Six investigator sites in France (Bordeaux, Montpellier, Nancy, Paris, Strasbourg and Toulouse) where recruitment is open.

TITAN trial (phase I) NCT06916806

France and abroad

72 (18 to 70 years old)

May 2025 – June 2027
1 year of follow-up

Subcutaneous

DR-0201

- Phase I, open-label trial (NCT06647069) taking place in several countries (not in France) and involving 62 adults with various autoimmune diseases, including dermatomyositis and polymyositis.

Other drugs

⇨ Interleukin-2

Interleukin 2 (IL-2) is an inflammatory mediator that:

- exerts a beneficial effect on the balance and function of regulatory T cells (Treg) in the blood,
- may therefore be beneficial in the treatment of various autoimmune diseases, including dermatomyositis.

In China, a phase III trial (NCT05495321) is aiming to recruit 240 participants in order to compare the effects of low doses of IL-2 to those of a placebo in dermatomyositis.

⇨ Tocilizumab

NEW

- Tocilizumab binds specifically to interleukin-6 (IL-6) receptors. IL-6 is another inflammatory mediator involved in T cell activation and the secretion of immunoglobulins (which include autoantibodies).
- In China, the open-label PROMISE-MDA5 trial (NCT07377058) is aiming to recruit 110 adults with dermatomyositis in order to evaluate tocilizumab.

⇨ Omega-3 fatty acid supplementation

NEW

Like other types of myositis, dermatomyositis is thought to be caused by a combination of:
- a genetic predisposition (multiple genes involved),
- "environmental" factors in a broad sense, particularly those linked to lifestyle and diet.

Learn more

Fatty acids are fat-like substances (lipids) found in certain foods. Omega-3 fatty acids are known as "essential" fatty acids as they must be obtained from food (the body cannot make them), yet they support heart and vascular health, brain function, etc.
In the United States, a phase II trial (NCT07111065) is aiming to recruit 300 adults with dermatomyositis. They will take omega-3 fatty acid capsules or a placebo for six months and will receive dietary coaching on adopting a more balanced diet.

⇨ Sodium thiosulfate for calcinosis

Up to 75% of individuals with juvenile dermatomyositis have abnormal collections of calcium deposits (calcifications) under their skin and in other tissues known as calcinosis.

In France, a trial:
- is evaluating sodium thiosulfate injected into calcifications in several diseases (including dermatomyositis),
- is taking place at eight investigator sites.

ITS-PILOT trial (phase II) NCT03582800

France

40
(over 6 months old)

Jan 2020 – June 2027
1 year of follow-up

Injected into calcifications

In the United States, a phase II, open-label trial (NCT06672822) is evaluating the same treatment in 20 adults with dermatomyositis or another autoimmune disease.

Specific observational studies and databases

⇨ In France

- Study conducted at **CHU de Strasbourg** [Strasbourg University Hospital] in children and adults with dermatomyositis that led to panniculitis (inflammation of fatty tissue).
- To identify characteristics (visual, histological) that would distinguish it from panniculitis caused by something else.

PANNICUL observational study NCT07345949

NEW

France

20
(over 1 year old)

Oct 2025 – April 2028
2.5 years of follow-up

- Study conducted by **CHU de la Guadeloupe** [Guadeloupe University Hospital] in Caribbean dermatomyositis patients.
- To better understand why the disease is still associated with high morbidity and mortality in the French Caribbean.

DM-ANTILLES study NCT07265999


France


10
(over 16 years old)


July 2021 – July 2029
2 years of follow-up

⇨ In the United States

Studies are taking place in order to:

▪ analyse dietary habits and their impact on various diseases, including dermatomyositis and polymyositis (NCT06339957),

▪ improve our understanding and treatment of several diseases, including juvenile dermatomyositis (NCT00059748 - 757 participants).

Results helping to improve our understanding and management of dermatomyositis

⇨ Tryptophan

▪ Tryptophan is an amino acid found in various foods that the body uses to make serotonin (a hormone that helps regulate mood which is sometimes referred to as the "feel good hormone").

▪ Children with juvenile dermatomyositis are more at risk of developing anxiety or depression and have low levels of tryptophan and serotonin in their blood, according to a study whose results were published in 2026.

▪ Type I interferon, which is overproduced in dermatomyositis, seems to divert tryptophan towards another metabolic pathway (and not serotonin production).

▪ A study found that this phenomenon correlated with muscle inflammation and mental health test results, and that it could be inhibited *in vivo* (in patient cells) by two drugs called baricitinib and anifrolumab. *Wu Y et al. 2026*

⇨ Caution regarding the use of UV nail lamps

▪ A group of doctors in the United States reported the first known case of a young woman who developing dermatomyositis after applying nail varnish that required the use of a UV lamp.

▪ Caution is advised pending further studies, especially given that the use of these UV lamps has already been linked to cases of skin cancer, and that alternatives exist (air drying, LED lamps). *On A et al. 2025*

⇨ Skin colour can delay diagnosis

▪ Dermatomyositis is often more difficult to detect in darker skin tones where it manifests as a darkening or lightening of the skin's usual colour (rather than a reddish purple discolouration) and changes around the nails that are harder to see.

▪ This delays diagnosis and increases the risk of an initial misdiagnosis (which occurs in one in 10 patients).

Akuffo-Addo E et al. 2025 *Cantu-Martinez V et al. 2026*

⇨ Juvenile and adult dermatomyositis - comparable severity at diagnosis

▪ Whether dermatomyositis starts during childhood (juvenile dermatomyositis) or adulthood, it is no more likely to be severe at diagnosis (44% of cases) according to a study conducted in France involving 201 patients.

▪ In the cases where the disease was severe at diagnosis, interstitial lung disease was more common in the adults, and gastrointestinal vasculitis was more common in the children.

▪ The type of autoantibody detected was important. Anti-NXP2 autoantibodies were more frequently associated with severe disease at diagnosis in the juvenile form. In the adult form, anti-Mi2 autoantibodies were associated with a lower risk of severe disease.

Sinnaeve A et al.2026

In immune-mediated necrotising myopathy

What's the problem?

- Immune-mediated necrotising myopathy was recognised as a distinct type of myositis relatively late, only appearing in classification schemes for inflammatory myopathies in 2003.
- Drugs used to treat it are similar to those prescribed for dermatomyositis, however, as with dermatomyositis, they are sometimes not effective enough or cause significant adverse events.

Good to know
In France, **nearly 700** people are monitored by a specialist centre for immune-mediated necrotising myopathy according to data collected by the BNDMR.
BNDMR national report. February 2026 [report available in English]

What treatments are currently in trials?

Various clinical trials recruit individuals with immune-mediated necrotising myopathy, but they are rarely studying that disease specifically (they may also recruit participants with other autoimmune diseases).
For more information:
⇨ See chapter on innovative non-pharmacological treatments in trials
⇨ See chapter on trials in several types of myositis

⇨ Ublituximab (Briumvi®)

NEW

How does it work?
Ublituximab is:
- an antibody directed against CD20, a protein found on the surface of B cells (precursors of the cells that secrete the autoantibodies involved in immune-mediated necrotising myopathy (anti-SRP, anti-HMGCR))
- already approved for the treatment of adults with multiple sclerosis (another autoimmune disease).

Learn more [French]

It should be noted that various publications have previously reported on the efficacy of rituximab (another CD20-directed antibody) in refractory immune-mediated necrotising myopathy.

The SUMMIT trial
- Placebo-controlled trial of ublituximab in early (onset within one year prior to inclusion), active, autoantibody-positive (anti-SRP, anti-HMGCR) immune-mediated necrotising myopathy.
- Recruiting, taking place at eight investigator sites in the United States.

SUMMIT trial (phase II) NCT07103746

United States	30 (over 18 years old)	Feb 2026 – Aug 2029 1.5 years of follow-up	Intravenous

⇨ Immunoglobulins

How do they work?
Pooled human immunoglobulins (Ig) are antibodies taken from healthy donors which are able to modulate immune system activity (immunomodulatory effect) in myositis.

In the United States, the phase II MIGHT trial (NCT06599697) is exploring the efficacy, safety and tolerability of infusions of Ig or placebo in 12 anti-HMGCR immune-mediated necrotising myopathy patients over the age of 16 until the end of 2026.

Efgartigimod - another treatment avenue

⇨ How does it work?

- Immunoglobulin G (IgG) antibodies are the main type of antibody produced by the immune system. These include anti-SRP and anti-HMGCR autoantibodies, which are specific to immune-mediated necrotising myopathy.
- Neonatal Fc receptors (FcRn) bind to IgG antibodies, preventing them from being degraded. In doing so, they help extend the time that IgG antibodies circulate in the blood.
- Conversely, FcRn blockers like efgartigimod enhance the degradation of Ig, leading to a reduction in autoantibodies.
- Efgartigimod (Vyvgart®) has already been approved in France for the treatment of refractory forms of myasthenia gravis.

⇨ What's new?

- A 2025 publication reported the results of early efgartigimod use (without waiting for other treatments to fail) in three patients with anti-SRP immune-mediated necrotising myopathy.
- One injection of efgartigimod per week for one month led to significant improvements in muscle strength and creatine kinase (CK) levels. *Peng Q et la. 2025*

Evaluated in various types of myositis
In several countries around the world (including France), efgartigimod is being evaluated in various types of myositis, including immune-mediated necrotising myopathy (ALKIVIA trial or NCT05523167, see page 27).

New information to help us better understand and manage the disease

⇨ Genes and bacteria

The immune system dysfunction that occurs in immune-mediated necrotising myopathy is thought to be caused by a combination of predisposing genetic factors and environmental factors (infections, drugs, etc.) that have yet to be determined. This year:

- researchers identified 128 sequences of DNA (a molecule arranged into chromosomes that contains the genetic code for an organism) whose variations could play a role in the genetic predisposition to developing immune-mediated necrotising myopathy,
- another team reported the first known case of an individual with tuberculosis-induced immune-mediated necrotising myopathy, with anti-tuberculosis drugs treating both the diseases effectively. *Lin Z et al. 2026* *Colpani A et al. 2025*

⇨ A sometimes atypical clinical presentation

- Immune-mediated necrotising myopathy can manifest in unusual ways (chronic and slowly progressive muscle weakness, isolated swallowing difficulties (dysphagia), eye muscle involvement, asymptomatic increase in CK levels).
- This can result in misdiagnosis (limb-girdle muscular dystrophy, facioscapulohumeral muscular dystrophy, etc.) which delays the implementation of effective treatment.

Skolka MP et al. 2025

⇨ An alternative to statins?

- Statins (drugs taken to lower cholesterol levels) can trigger anti-HMGCR immune-mediated necrotising myopathy.
- In the event of statin-induced anti-HMGCR immune-mediated necrotising myopathy, statin use should be discontinued.
- Another cholesterol-lowering drug called bempedoic acid may be able to be used instead.
- In a study involving 10 patients, when taken for six months, bempedoic acid lowered cholesterol levels and no disease recurrence was observed. *Benucci Met al.2025*

⇨ Anti-HMGCR autoantibodies toxic to muscle fibres

Learn more [French]

In 2019, a French team (Hôpital Pitié-Salpêtrière) supported by AFM-Téléthon showed that autoantibodies specific to immune-mediated necrotising myopathy (anti-SRP, anti-HMGCR) were not just biomarkers of the disease.

- They also play a direct role in the onset of the disease, causing muscle fibre atrophy and impairing muscle regeneration mechanisms.

Learn more [French]

- In October 2025, these same researchers (in collaboration with researchers from Strasbourg) published findings on the role of anti-HMGCR autoantibodies in muscle damage. These autoantibodies inhibit the HMGCR enzyme, causing muscle cells to fail to properly metabolise certain fatty acids. Lipid droplets accumulate in their cytoplasm, which is accompanied by muscle fibre necrosis. *Giannini M et al. 2025*

In antisynthetase syndrome

What's the problem?

Learn more [French]

- Antisynthetase syndrome causes symptoms in the lungs (interstitial lung disease), muscles (myositis) and joints.
- Some respiratory problems progress very quickly, while others progress much more slowly, however, no criteria have been identified to distinguish between them at the outset.
- International diagnostic criteria for antisynthetase syndrome were only published in 2023, and its treatment has not yet been standardised.

Good to know
In France, **nearly 1,300** people are monitored by a specialist centre for antisynthetase syndrome according to data collected by the BNDMR.
BNDMR national report. February 2026 [report available in English]

Are there any clinical trials currently taking place?

⇨ Stem cell-derived exosomes

- Mesenchymal stem cells have the ability to differentiate into different types of cells to repair tissue, but they also have immunomodulatory and anti-fibrotic properties.
- They release tiny vesicles called exosomes into their environment which can have beneficial effects on nearby cells.

A kind of "stem cell juice"
With the support of AFM-Téléthon, Prof. Philippe Menasché from the Hôpital Européen Georges Pompidou [Georges Pompidou European Hospital] in Paris is working on using repair factors produced by stem cells to treat heart muscle diseases.
Interview with Prof. Menasché recorded during the French Telethon [video in French]

In China, a trial (NCT06919380) is evaluating nebulised (aerosol) mesenchymal stem cell-derived exosomes (MSC-exos-P1) in 10 adults with anti-MDA5 dermatomyositis and interstitial lung disease.

⇨ A comparison of two treatments

NEW

The JAKCNIASSILD trial (NCT07406932) is aiming to recruit 80 adults with antisynthetase syndrome in China in order to evaluate initial therapy with either:

- a JAK inhibitor (tofacitinib, baricitinib or upadacitinib),
- or a type of immunosuppressant called a calcineurin inhibitor (cyclosporin or tacrolimus).

⇨ Emapalumab (Gamifant®)

NEW

- Emapalumab is an antibody that neutralises interferon-gamma (a mediator of immunity).
- A trial (NCT07486869) in the United States is planning to evaluate it in five adults with anti-MDA5 antibody positive rapidly progressive interstitial lung disease.

⇨ Tacrolimus, tofacitinib and thalidomide

In China, a trial (NCT06438679) is currently evaluating tofacitinib (a JAK inhibitor), thalidomide (an immunosuppressant) and tacrolimus in 133 adults with anti-MDA5 dermatomyositis and respiratory impairment.

Observational studies to gain a better understanding of the disease

⇨ What's new?

Results from the French TYPASS study

- An analysis of the medical records of 132 adults with antisynthetase syndrome monitored at seven expert centres in the north-east of France showed that 39% had rapidly progressive interstitial lung disease.
- Signs and symptoms associated with this diagnosis were fever, "organising pneumonia" and pleural effusion (the accumulation of excess fluid in the space between the lungs and the chest wall).
- In the future, the detection of these three characteristics when interstitial lung disease is diagnosed could prompt closer monitoring or even more intensive treatment.

Billotte M et al. 2025

⇨ Another study in France

- The CYTILDASS study involving 24 adults with newly-diagnosed antisynthetase syndrome is taking place in six investigator sites in France.
- Its objective is to look for a correlation between the number of Th1 and Th17 cells (helper T cells) present in the lungs when interstitial lung disease is diagnosed and its clinical course.

CYTILDASS study NCT05984394

France	24 (over 18 years old)	Started in Aug 2024 6 months of follow-up

⇨ Studies outside France

- In Norway, the RMD-mILDer study (NCT06732674) is comparing the results of home monitoring (measuring vital capacity, oxygen saturation, temperature, a questionnaire completed by patients, etc.) with those of traditional monitoring at a hospital for one year.
- In the United States, an observational study (NCT01276470) which plans to recruit 580 participants, is attempting to confirm the hypothesis that environmental factors (microorganisms, smoking, toxins, ultraviolet radiation, etc.) increase the risk of developing antisynthetase syndrome.
- In China, an observational study (NCT06203249) is analysing the microorganisms present in the lungs (bronchoalveolar lavage fluid microbiota) of patients with anti-MDA5 dermatomyositis and interstitial lung disease in order to look for a relationship between the composition of this microbiota and interstitial lung disease.

In several different types of myositis

What's the problem?

- Some myositis patients have contraindications to conventional treatments. For others, these drugs cause severe side effects or they are not effective enough, or not effective at all.
- In all of these situations, finding new treatments is essential. "Basket trials" make it possible to evaluate the same drug candidate in several types of myositis at the same time.

What is a basket trial?

- Basket trials aim to demonstrate the benefits of a single drug candidate in a number of diseases or disease subtypes. They recruit participants with different diseases or disease subtypes but who share a common link.
- This type of approach is mainly used in oncology to test cancer drugs that target specific biological abnormalities shared by tumours in various different organs or tissue types (brain, pancreas, breast, etc.). It is also used in immune system disorders, with basket trials covering various autoimmune diseases (lupus, scleroderma, myositis, etc.) or even different types of myositis (dermatomyositis, immune-mediated necrotising myopathy, etc.) as they share common immune mechanisms.

SFPT.org [page in French]

Basket trials currently taking place

Name	Trials	In France	Stage	Key points
Efgartigimod (Vyvgart®)	NCT05523167 ALKIVIA NCT05979441 ALKIVIA+	Yes	II/III	• FcRn blocker • MA for refractory myasthenia gravis • infusion or subcutaneous injection • not recruiting
Nipocalimab (Imaavy®)	NCT05379634 SPIREA	Yes	II	• FcRn blocker • MA for refractory myasthenia gravis • infusion • not recruiting
RAY121 NEW	NCT06371417 RAINBOW NCT06723106 RAINBOW-LTE	Yes	I	• anti-complement antibody • subcutaneous injection • recruiting
Filgotinib (Jyseleca®)	NCT06285539 DRIMID	No	II	• Janus kinase inhibitor • tablets • recruiting

⇨ Efgartigimod and nipocalimab - two FcRn blockers

How do they work?

- FcRn bind to antibodies (and autoantibodies), preventing them from being degraded. In doing so, they help extend the time that autoantibodies circulate in the blood, and therefore prolong the manifestations of autoimmunity.
- FcRn blockers (anti-FcRn) block FcRn with the aim of limiting the lifespan of autoantibodies.

Who are they for?

They are being tested in patients whose myositis remains active despite treatment.

Clinical trials currently taking place

Efgartigimod (Vyvgart®)

- Placebo-controlled trial (ALKIVIA) followed by open-label trial (ALKIVIA+ - all participants receive efgartigimod) involving patients with dermatomyositis, polymyositis, immune-mediated necrotising myopathy or antisynthetase syndrome.
- Four investigator sites in France (Lyon, Paris, Rouen and Strasbourg).

ALKIVIA trial (phase II/III)			NCT05523167
France and abroad	265 (over 18 years old)	Oct 2022 – Feb 2027 1 year of follow-up	Subcutaneous

ALKIVIA+ trial (phase III)			NCT05979441
France and abroad	240 (over 18 years old)	Sept 2023 – Sept 2028 14 months of follow-up	Subcutaneous

Nipocalimab (Imaavy®)

- Trial evaluating nipocalimab initially in a placebo-controlled format, then open-label, in patients with dermatomyositis, immune-mediated necrotising myopathy or antisynthetase syndrome.
- Three investigator sites in France (Nice, Paris and Strasbourg).

SPIREA trial (phase II)			NCT05379634
France and abroad	36 (over 18 years old)	July 2022 – Sept 2026 Up to 2 years of follow-up	Intravenous

⇨ RAY121 - an anti-complement antibody

How does it work?

Complement comprises several proteins involved in immune reactions. RAY121 is a monoclonal antibody directed against complement component 1 (C1).

Who is it for?

Patients whose disease is resistant or intolerant to corticosteroids and/or immunosuppressants.

What's new?

Results from preclinical animal studies demonstrated RAY121's ability to neutralise C1 for an extended duration, with the potential for a single monthly injection. *Ho AWS et al. 2025*

Clinical trials currently taking place

- Open-label trial (no placebo) in multiple autoimmune diseases, including two types of myositis (dermatomyositis and immune-mediated necrotising myopathy).
- Two investigator sites in France (Montpellier and Paris).

RAINBOW trial (phase I) NCT06371417

France and abroad	144 (18 to 85 years old)	Aug 2024 – June 2026 7.5 months of follow-up	Subcutaneous

- Long-term extension of the RAINBOW trial.

NEW

RAINBOW trial (phase I) NCT06723106

France and abroad	144 (18 to 85 years old)	Dec 2024 – April 2026 1 year of follow-up	Subcutaneous

⇨ Filgotinib (Jyseleca®) - a JAK inhibitor

- Filgotinib inhibits JAK (enzymes needed to activate the signalling pathways involved in chronic inflammation).
- In the Netherlands, the phase II DRIMID trial (NCT06285539) is aiming to recruit 60 adults with various different diseases, including dermatomyositis and antisynthetase syndrome.

New data supporting the implementation of exercise

Learn more [French]

The results of two studies were published in recent months:

- One study involving 23 individuals recently diagnosed with myositis showed that a three-month high-intensity interval training programme (three sessions a week) increased muscle endurance more than a low- to-moderate-intensity programme (five sessions a week), with no increase in disease activity. *Andreasson KM et al. 2025*

- Another study involving 15 myositis patients demonstrated that the benefits of a supervised, personalised high-intensity resistance training programme (one hour twice a week for four months) on endurance and quality of life were still present one year after starting the programme, with no increase in disease activity. *Jensen KY et al. 2025*

Databases and observational studies

⇨ What else has been happening?

A project is underway in France to create a national database dedicated to inflammatory myopathies (**BASE Myosites**). This database will be supported by the BNDMR and supplied with data by specialist centres for rare diseases.

⇨ In France

- The MASC2 database is a national database led by the Groupe Hospitalier Pitié-Salpêtrière [Pitié-Salpêtrière Hospital Group] in Paris which collects data and samples (blood and/or muscle) from adult myositis patients.

MASC2 database NCT05454527

France	3,200 patients	Since 2013

▪ The MAIA database was created by CHU de Brest [Brest University Hospital] and collects clinical data and biological samples (blood, muscle, etc.) from 60 individuals with suspected myositis.

MAIA database NCT04792931

France | 60 patients (over 18 years old) | Created in 2022

▪ CHU de Strasbourg [Strasbourg University Hospital] is conducting an observational study on medical data from adult myositis patients with renal involvement monitored between 1999 and 2023.

Renal manifestations study NCT06904937

France | 15 (over 18 years old) | Started in Jan 2024, 14 months of follow-up

⇨ Outside France

▪ The EuroMyositis database brings together various different registries and databases and collects data from over 3,000 myositis patients www.euromyositis.eu

▪ Various large-scale observational studies are currently taking place around the world (NCT00017914, NCT05738824, NCT01171573, NCT02468895).

NEW

An online survey (in English)

The international MIHRA foundation is conducting an observational study (NCT07374107) which was launched at the request of those affected in order to identify the most pressing research concerns expressed by patients (or their parents for minors).

New developments in the disease, diagnosis and monitoring

⇨ Towards a new classification system structured around autoantibodies

In an article published in May 2026, two French experts (Prof. Olivier Benveniste and Prof. Yves Allenbach) distinguished five main types of myositis:

- inclusion body myositis,
- immune-mediated necrotising myopathy,
- dermatomyositis,
- overlap myositis,
- antisynthetase syndrome.

▸ The first two primarily affect muscles. The other three can affect multiple systems (skin, joints, lungs).

▸ Polymyositis is now considered to be very rare. Many patients who were diagnosed with polymyositis in the past would now be diagnosed with overlap myositis. Conversely, antisynthetase syndrome is now classified as a distinct entity, rather than being grouped with overlap myositis.

▸ Each type of myositis has different mechanisms which are becoming increasingly more understood, enabling more tailored treatments to be developed.

▸ Autoantibodies now play a key role in diagnosis and monitoring. Each type is associated with particular manifestations (muscular, cutaneous, etc.) and histological features (seen on biopsies). When they are present (70% of patients), they make it possible to make a diagnosis, determine the type of myositis and get a sense of the disease's course.

▸ Various myositis subtypes have been identified in recent years. Each one is associated with a specific type of autoantibody (anti-SRP or anti-HMGCR for immune-mediated necrotising myopathy, anti-TIF1-gamma or anti-MDA5 for example for dermatomyositis, etc.). These subtypes have different courses and often different sensitivities to certain drugs. *Allenbach Y et al. 2026*

⇨ Autoantibody test results still vary from one laboratory to another

Fifteen laboratories located in Europe, North America, Australia and Japan received samples from 24 patients to test for myositis-associated autoantibodies and myositis-specific autoantibodies:

- the autoantibodies detected varied from one laboratory to another,
- the laboratories used different assays,
- those using the same assays obtained different results,
- anti-OJ, anti-EJ and anti-TIF1-gamma antibodies were not reliably detected.

The authors of this study call for the urgent adoption of measures to harmonise practices across laboratories. *Harvey GR et al. 2026*

⇨ A distinct form of myositis triggered by a cancer drug

A genetic predisposition?

- Immune checkpoint inhibitors, which are used to treat several types of cancer, can cause myositis and myocarditis. A study found that this phenomenon was more common in individuals with the HLA-A*01:01-B*08:01-C*07:01 genetic variant. This observation needs to be confirmed but could be used to identify patients at risk of myositis or myocarditis before starting immune checkpoint inhibitors. *Müller-Jensen L et al. 2025*

An international consensus

Learn more

In March 2026, experts from various counties met at a European Neuromuscular Centre (ENMC) workshop organised by Prof. Yves Allenbach (Paris) to create guidelines on the diagnosis and treatment of immune checkpoint inhibitor-induced myositis.

⇨ Caution regarding the use of certain herbal dietary supplements

- An analysis of studies on herbal dietary supplements (herbal medicine) identified 15 supplements that have demonstrated immunostimulatory effects.
- These included spirulina, ginseng, garlic and green tea extract. They are found in numerous dietary supplements used for self-medication and can trigger flare-ups in individuals with an autoimmune disease. *Weiner JD et al. 2025*

⇨ Myositis in Martinique

- According to a recent study, inflammatory myopathies are more common in Martinicans of African descent, the most common types being antisynthetase syndrome, dermatomyositis and overlap myositis. At diagnosis, 52% of the patients in the study had respiratory involvement (interstitial lung disease) and 16% had cardiac involvement.
- Despite well-managed treatment, 40% of the patients relapsed in a median time of three years. These patients therefore require rapid treatment and close monitoring.

Abel A et al. 2025

⇨ Mixed results for two exoskeletons

Learn more

- With the support of AFM-Téléthon, the Institut de Myologie (Paris) evaluated two lower limb exoskeletons called MyoSuit and Keeogo in 28 patients (13 of whom had inclusion body myositis or dermatomyositis).
- The exoskeletons reduced the patients' sit-to-stand and walking performances (speed, stride length, cadence).
- While researchers welcome the promises offered by these devices, they also emphasise the need for finer tuning in order to be able to provide optimal assistance to individuals with significant muscle weakness. *Feigean R et al. 2026(1)* *Feigean R et al. 2026(2)*

⇨ Using artificial intelligence

▪ **To produce health-related content:** ChatGPT and DeepSeek's abilities to create brochures for patients on various diseases (including dermatomyositis) were found to be similar, but still limited ("moderate" accuracy and reliability, content sometimes difficult to understand). These shortcomings justify using them as a complement to documents validated by experts, and not as a replacement.

▪ **To create exercise programmes**: 12 specialist physiotherapists (including six from Europe) described ChatGPT's exercise recommendations for inclusion body myositis as safe and accurate, but lacking in comprehensiveness. In another study, ChatGPT's responses to 70 frequently asked questions regarding exercise and rehabilitation in adolescents with myositis were deemed to be accurate and fairly reliable, but difficult for the general public to understand.

▪ **To analyse chest CT scans**

Myositis-associated interstitial lung disease is detected using chest CT scans. When analysing CT scans from 107 patients, an AI-based tool was able to reliably detect certain (inflammatory) lesions, but should still only be used as a complement to interpretation by a radiologist.
Alluri AA et al. 2025 *Schütze K et al. 2026* *Sari F et al. 2026* *Kuzmanovic Y et al. 2026*

⇨ Falls and fractures

A study involving 470 myositis patients (all types) over the age of 18 found that:
▪ 80% had had a fall since their diagnosis, and 57% had fallen within the past year,
▪ inclusion body myositis was associated with the highest risk of falling,
▪ 25% had had fall-related fractures since their diagnosis, with half of these patients experiencing at least two fractures and one third needing surgery.

Learn more [French]

There are ways to prevent falls and reduce the risk of fractures (taking calcium, vitamin D, bisphosphonates, etc.) but they are relatively underused. *Nandakumar D et al. 2026*

⇨ The involvement of mitochondrial abnormalities

Mitochondrial dysfunction was already known to exist in inclusion body myositis. A study conducted in 850 patients by researchers (including those from the Institut de Myologie in Paris) showed that these abnormalities are also present in other types of myositis. They might even play a role in the development of inflammatory myopathies, which would open up new treatment avenues. *Lauletta A et al. 2025* *Torri F et al. 2026*

⇨ Cardiac involvement is common

A study conducted in 34 patients newly diagnosed with myositis (compared to nine healthy individuals) showed that:
▪ cardiac involvement (myocarditis and/or pericarditis) was detected by cardiac MRI in nearly half of the patients at the time of diagnosis,
▪ it was often silent (no symptoms) and benign,
▪ it was closely linked to the severity of skeletal muscle involvement, suggesting a shared mechanism. *Hanna B et al. 2026*

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Keep up to date on myositis research news throughout the year on the AFM-Téléthon website:
www.afm-telethon.fr/en/latest-news