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obiettivo sanità salute
26-29 NOVEMBRE 2024
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Efficacia, sicurezza e tollerabilità dei Mabs Miastenia gravis

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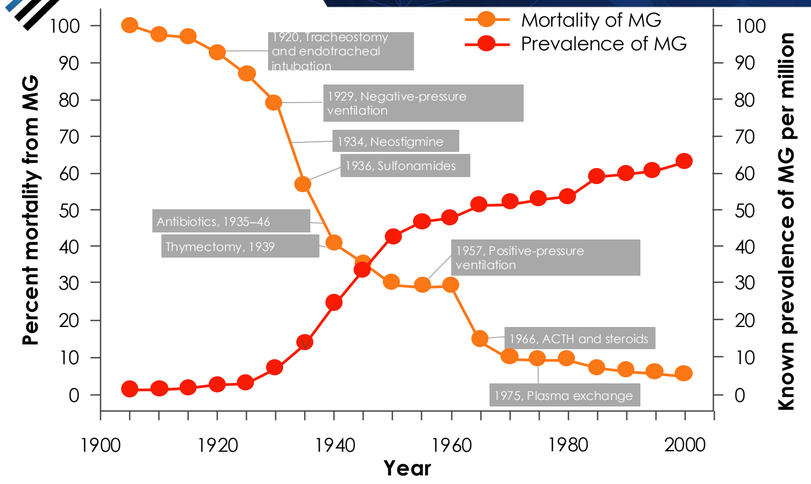
Immunotherapy in myasthenia gravis in the era of biologics

Marinos C. Dalakas[1,2]

Abstract | No consensus has been reached on the ideal therapeutic algorithm for myasthenia gravis (MG). Most patients with MG require induction therapy with high doses of corticosteroids and maintenance with an immunosuppressant. Severe cases and acute worsening require intravenous immunoglobulin or plasmapheresis before oral immunosuppressants start having an effect. However, biologics are emerging as important therapeutic tools that promise to provide better corticosteroid sparing effects than standard treatments and can even induce remission.

NATURE REVIEWS | NEUROLOGY VOLUME 15 | FEBRUARY 2019 |



Negli ultimi decenni la prevalenza è in costante aumento.

Si stima al momento una prevalenza di circa 11-36 casi per 100000 abitanti e un'incidenza di circa 10-29 casi per milione di abitanti per anno in Europa

Aumento prevalenza dovuto a:

-riduzione mortalità

-miglioramento diagnosi

-invecchiamento popolazione

Adapted from Grob D, et al. Muscle Nerve. 2008;37:141–149.

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- **225,8/mil ab (stima Toscana: 842 casi (Finocchietti et al., 2024)** Diagnosi di dimissione o esenzione

- **Casi stimati (Antonini et al., 2023): Toscana 503-1092 (**esenzione, dimissione, almeno una piridostigmina)

- **Dati del Registro Malattie Rare Toscana**

Residenti in vita con esenzione RFG101 a novembre 2023: 1820 (**prevalenza 488/mil ab**)

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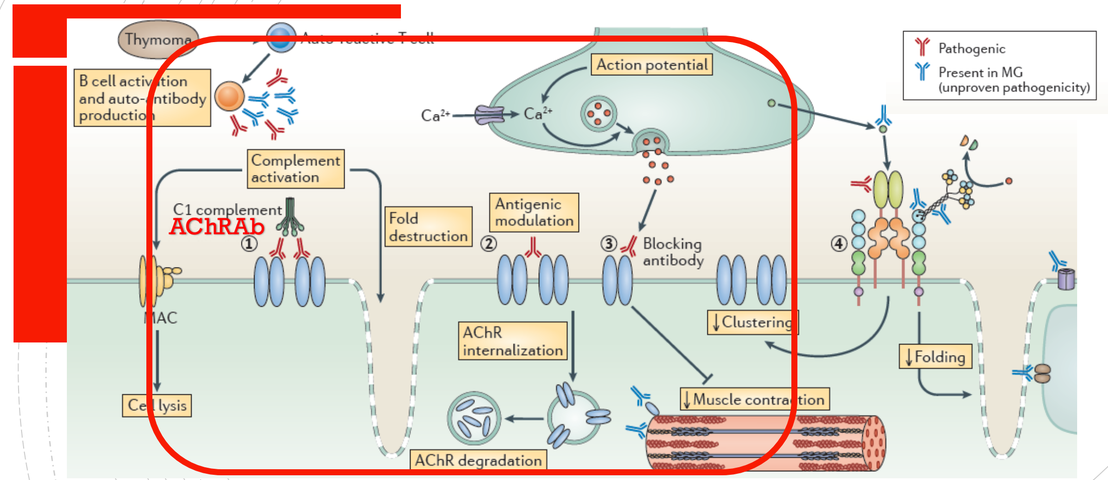

Gilhus NE et al. Nat Rev Neurol. 2016 May;12(5):259-68

Caratteristiche peculiari dei diversi sottogruppi di Miastenia

Table 1 | **Characteristics of myasthenia gravis subgroups**

Subgroup	Antibody	Additional antibodies	Age at onset	Proportion of patients (%)	Thymus	Clinical benefit of thymectomy
Early-onset	Anti-AChR	Rare	<50 years	15–25	Hyperplasia common	Proven
Late-onset	Anti-AChR	Common	>50 years	35–45	Atrophy common	Not proven (but possible)
Thymoma	Anti-AChR	Very common	Any	10	Lymphoepithelioma	Proven
MuSK	Anti-MuSK	Rare	Any	1–10	Normal	None
LRP4	Anti-LRP4	Rare	Any	1–5	Normal	None
Seronegative	None of the above detected	Variable	Any	10–15	Variable	None
Ocular	Variable	Rare	Any	15	Variable	None

AChR, acetyline choline receptor; LRP4, low-density lipoprotein receptor-related protein 4; MuSK, muscle-specific kinase.

Juvenile MG <18 yrs

Traditional therapeutic options

Decades introduced:

1930s:	physostigmine & neostigmine
1930s & 1940s:	thymectomy
1950s:	mechanical ventilation, edrophonium chloride, & phridostigmine
1960s:	corticosteroids & plasmapheresis
1960s & 1970s:	azathioprine
1980s:	cyclosporine
1980s & 1990s:	IVIg
1990s & 2000s:	mycophenolate mofetil
2000s	rituximab, metotrexate


Dr Mary Walker (left) with patient Dorothy Codling (right) at St Alfege's Hospital, Greenwich in 1934)

Grob et al., 2008

Off label

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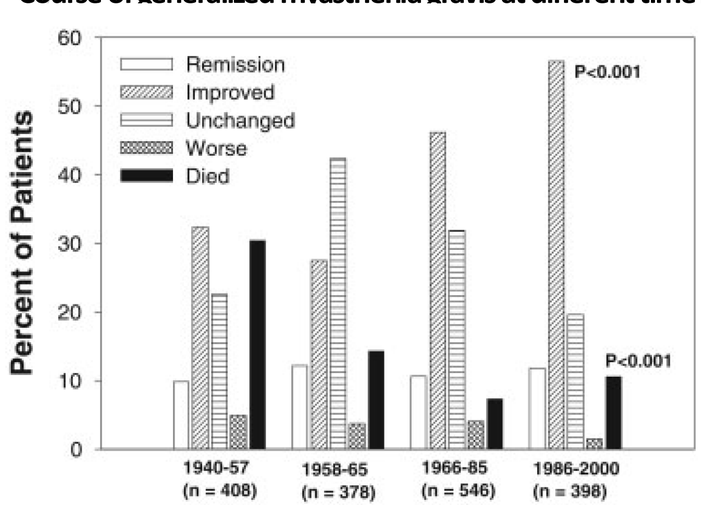
Course of generalized myasthenia gravis at different time periods



Grob et al., 2008

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Seminars in Arthritis and Rheumatism 55 (2022) 152010
Contents lists available at ScienceDirect
Seminars in Arthritis and Rheumatism
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The glucocorticoid toxicity index: Measuring change in glucocorticoid toxicity over time

John H. Stone*, P. Jane McDowell, David R.W. Jayne, Peter A. Merkel, Joanna Robs
Naomi J. Patel, Yuqing Zhang, Huibin Yue, Pirow Bekker, Liam G. Heaney
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Side effects and treatment burden

Table 2
GTI Domains Assessed Through Simple Clinical Measures or Laboratory Tests.

Domain	Description
Body Mass Index	Height, weight
Blood Pressure*	Systolic and diastolic blood pressures
Glucose Metabolism*	Hemoglobin A1c
Lipid Metabolism*	Low-density lipoprotein
Bone Mineral Density	Dual X-ray absorptiometry (DEXA scan)
Glucocorticoid Myopathy	Physical examination testing for proximal muscle weakness
Skin Toxicity	Physical examination
Neuropsychiatric Effects	Patient interview
Infection	Adverse events reporting

* Increases and decreases in medications for hypertension, glucose metabolism, and hyperlipidemia are considered in the GTI scoring algorithm.

Unresponsiveness to treatment

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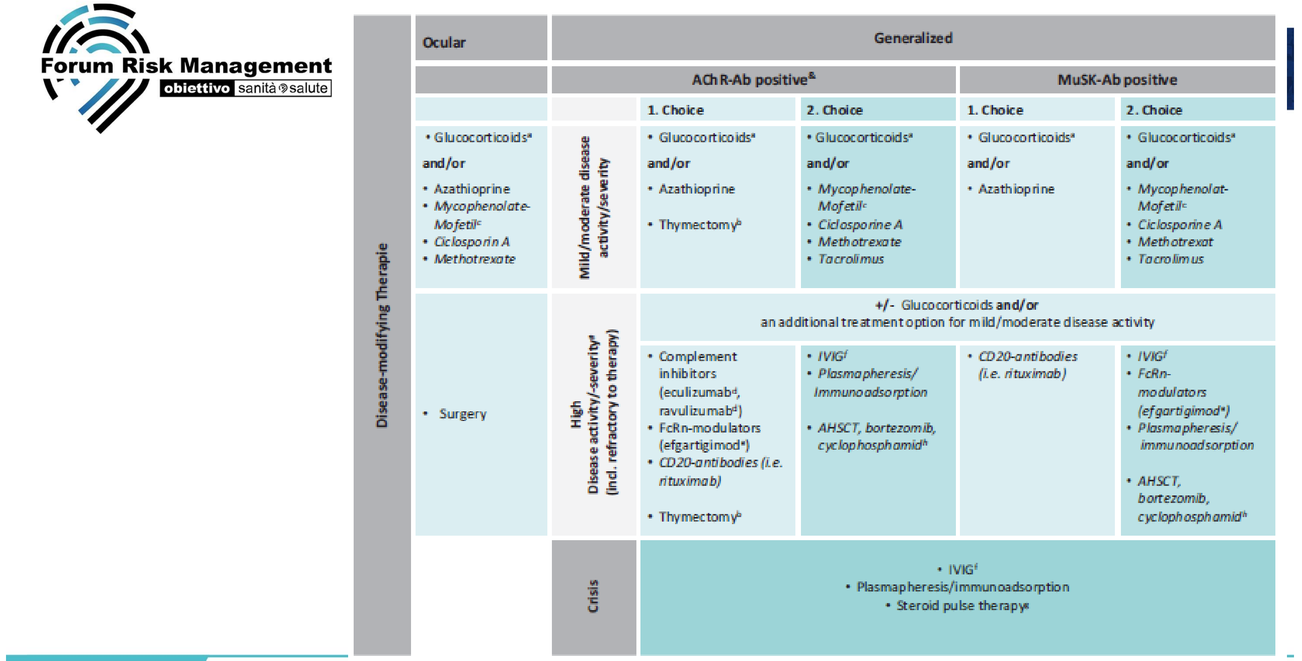
Table 1 Clinical features of 677 patients with myasthenia gravis included in the study[a]

	AChR+	MuSK+	DN	*p* Value[b]
No. of subjects	517 (76.4)	55 (8.3)	105 (15.7)	
Sex				0.016
Female	342 (66.1)	46 (83.6)	77 (73.3)	
Male	175 (33.9)	9 (16.4)	28 (26.7)	
Sex ratio, F:M	1.9:1	5.1:1	2.7:1	
Disease onset, y	37.7 ± 18.3	37.2 ± 15.1	36.3 ± 15.6	0.749
<40	313 (60.5)	32 (58.2)	60 (57.1)	0.784
≥40	204 (39.5)	23 (41.8)	45 (42.9)	
Disease duration, y	8.6 ± 6.4	6.6 ± 5.6	7.1 ± 6.1	0.012
Time to diagnosis, y	0.8 ± 1.6	0.5 ± 0.7	0.5 ± 0.9	0.326
Clinical stage at onset				<0.001
Ocular	180 (34.8)	16 (29.1)	45 (42.9)	
Generalized	154 (29.8)	5 (9.1)	35 (33.3)	
Bulbar	182 (35.2)	33 (60.1)	25 (23.8)	
Respiratory	1 (0.2)	1 (1.8)	—	
Clinical stage at max worsening				<0.001
Ocular	9 (1.7)	—	4 (3.8)	
Generalized	170 (32.9)	3 (5.5)	54 (51.4)	
Bulbar	303 (58.6)	46 (83.6)	46 (43.8)	
Respiratory	35 (6.8)	6 (10.9)	1 (1.0)	
Clinical stage at last observation				
CSR	115 (22.2)	2 (3.6)	23 (21.9)	0.005[c]
PR + O + G + B + R + D	402 (77.8)	53 (96.4)	82 (78.1)	
Pharmacologic remission	138 (26.7)	15 (27.3)	25 (23.8)	
Ocular	31 (6.0)	2 (3.6)	19 (18.1)	
Generalized	192 (37.2)	19 (34.6)	31 (29.5)	
Bulbar	30 (5.8)	16 (29.1)	7 (6.7)	
Respiratory	—	—	—	
Died of MG	11 (2.1)	1 (1.8)	—	
MGFA postintervention status				
CSR	115 (22.2)	2 (3.6)	23 (21.9)	0.003[d]
Positive outcomes (PR + MM + I)	245 (47.4)	35 (63.7)	44 (41.9)	
Negative outcomes (U + W + D)	157 (30.4)	18 (32.7)	38 (36.2)	
Pharmacologic remission	138 (26.7)	15 (27.3)	25 (23.8)	
Minimal manifestations	69 (13.4)	12 (21.8)	12 (11.4)	
Improved	38 (7.4)	8 (14.6)	7 (6.7)	
Unchanged	90 (17.4)	15 (27.3)	25 (23.8)	
Worse	56 (10.8)	2 (3.6)	13 (12.4)	
Died of MG	11 (2.1)	1 (1.8)	—	
Thymic surgery (n = 441)	364 (70.4)	25 (45.5)	52 (49.5)	
Age at surgery, y	35.5 ± 15.5	30.6 ± 9.9	33.5 ± 13.1	0.206
Histologic findings				<0.001

Baggi et al., Neurology, 2013



Disease-modifying Therapie						
	Ocular		Generalized			
			AChR-Ab positive[&]		MuSK-Ab positive	
			1. Choice	2. Choice	1. Choice	2. Choice
	• Glucocorticoids[a] and/or • Azathioprine • *Mycophenolate-Mofetil*[c] • *Ciclosporin A* • *Methotrexate*	Mild/moderate disease activity/severity	• Glucocorticoids[a] and/or • Azathioprine • Thymectomy[b]	• Glucocorticoids[a] and/or • *Mycophenolate-Mofetil*[c] • *Ciclosporine A* • *Methotrexate* • *Tacrolimus*	• Glucocorticoids[a] and/or • Azathioprine	• Glucocorticoids[a] and/or • *Mycophenolat-Mofetil*[c] • *Ciclosporine A* • *Methotrexat* • *Tacrolimus*
	• Surgery	High Disease activity/-severity[e] (Ind. refractory to therapy)	+/- Glucocorticoids **and/or** an additional treatment option for mild/moderate disease activity			
			• Complement inhibitors (eculizumab[d], ravulizumab[d]) • FcRn-modulators (efgartigimod*) • *CD20-antibodies (i.e. rituximab)* • Thymectomy[b]	• *IVIG*[f] • *Plasmapheresis/ Immunoadsorption* • *AHSCT, bortezomib, cyclophosphamid*[h]	• *CD20-antibodies (i.e. rituximab)*	• *IVIG*[f] • *FcRn-modulators (efgartigimod*)* • *Plasmapheresis/ immunoadsorption* • *AHSCT, bortezomib, cyclophosphamid*[h]
		Crisis	• IVIG[f] • Plasmapheresis/immunoadsorption • Steroid pulse therapy[g]			

Wiendl H. et al., Ther Adv Neurol Disord 2023

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Table 1 Summary of conventional immunosuppressive agents used in myasthenia gravis

Agent	Mechanism	Earliest time to clinical benefit	Dosing
Corticosteroids	Inhibition of T cells and monocyte-macrophage activation	2–12 wk	Initiation at 10/20 mg daily and weekly uptitration to 50/60 mg daily
Azathioprine	Purine analogue inhibiting DNA and RNA replication	12 mo	Initiation at 50 mg daily and increased weekl 2–3 mg/kg/d
Mycophenolate mofetil	Inhibition of inositol monophosphate dehydrogenase	6–12 mo	1–2 g/day in divided doses
Cyclosporine	Inhibits calcineurin	2–12 mo	Initiation at 3 mg/kg/d and increased to 6 mg titration based on clinical efficacy, therapeutic drug monitoring (400–600 ng/mL) and/or serum creatinine levels
Tacrolimus	Macrolide antibiotic that inhibits calcineurin	2–12 mo	3 mg/kg/d with further titration based on clinical efficacy or therapeutic drug monitoring (7–8 ng/mL)
Methotrexate	Folic acid antimetabolite	3–6 mo	Initiation at 10 mg/wk single dose, increased weekly up to 20–25 mg/wk
Cyclophosphamide	Alkylating agent preventing DNA replication	3–4 mo	Pulse of 1–1.5 mg/m^2 given over 5 d repeated monthly for 6 mo
IVIG	Multiple mechanism, predominantly FcRn saturation	10–15 d	2 g/kg divided over 2–5 d
SCIG	Same as IVIG but with lower peak and trough immunoglobulin levels and steadier state	2 wk	Weekly dose calculated by multiplying the maintenance dose of IVIG in grams by 1.37 divided by the interval between IVIG doses
PLEX	Removal of pathogenic antibodies by 'apheresis'	2–4 d	30–40 mL/kg of plasma exchanged per day for 5 d

d days, *DNA* deoxy ribonucleic acid, *FcRn* neonatal Fc receptor, *IVIG* intravenous immunoglobulin, *mo* month, *PLEX* therapeutic plasma exchange, *RNA* ribonucleic acid, *SCIG* subcutaneous immunoglobulin, *wk* week

[a] Off label

Time to effect

Drugs (2022) 82:861–887
https://doi.org/10.1007/s40265-022-01726-y
REVIEW ARTICLE

Pharmacotherapy of Generalized Myasthenia Gravis with Special Emphasis on Newer Biologicals

Deepak Menon[1] · Vera Bril[2]

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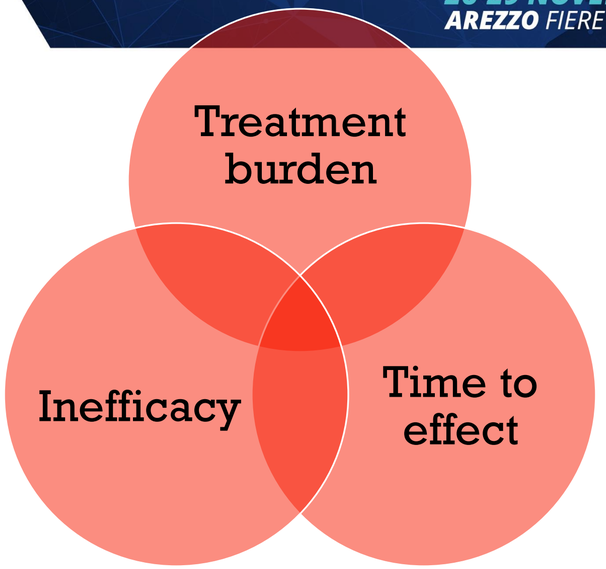
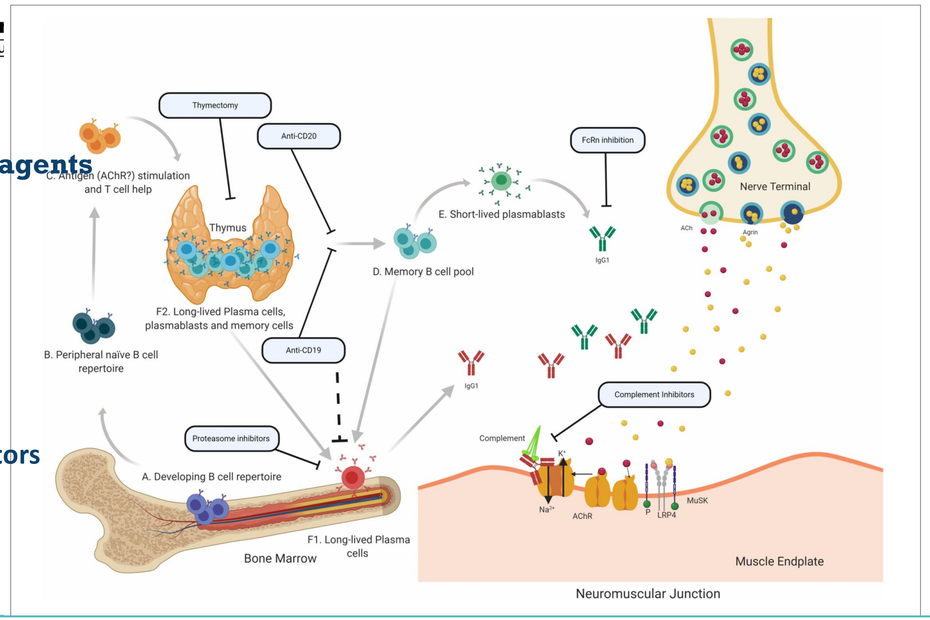
Unmeet needs



- **B cell-depleting agents**
  - Rituximab
  - Inebilizumab
- **FcRn inhibitors**
  - Efgartigimod
  - Rozanolixizumab
  - Batoclimab
  - Nipocalimab
- **Complement inhibitors**
  - Eculizumab
  - Ravulizumab
  - Zilucoplan



Fichtner et al., Front. Immunol., 2020

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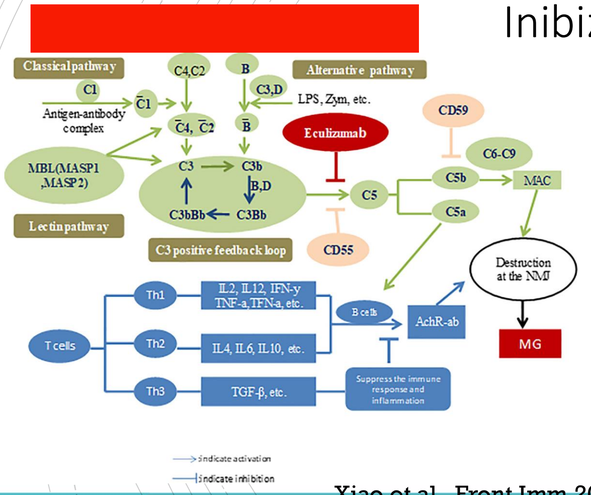
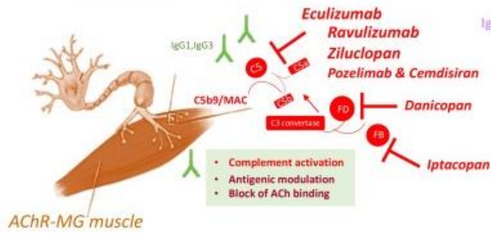
Inibizione del complemento



Xiao et al., Front Imm 2021



Cavalcante et al.,Front Imm, 2024

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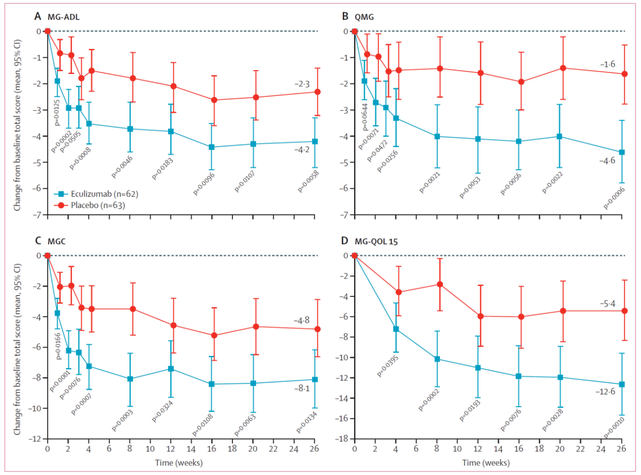
Safety and efficacy of eculizumab in anti-acetylcholine receptor antibody-positive refractory generalised myasthenia gravis (REGAIN): a phase 3, randomised, double-blind, placebo-controlled, multicentre study

*James F Howard Jr, Kimiaki Utsugisawa, Michael Benatar, Hiroyuki Murai, Richard J Barohn, Isabel Illa, Saiju Jacob, John Vissing, Ted M Burns, John T Kissel, Srikanth Muppidi, Richard J Nowak, Fanny O'Brien, Jing-Jing Wang, Renato Mantegazza, in collaboration with the REGAIN Study Group**

The schedule for eculizumab was induction dosing 900 mg on day 1 and weeks 1, 2, and 3; 1200 mg at week 4; and maintenance dosing 1200 mg every second week thereafter.



Drug name	Clinical trial	Mechanism	Dosing schedule	Treated participants (*n*)	Key efficacy measures (treatment vs placebo)	FDA approval for MG
Complement inhibitors						
Eculizumab	REGAIN[111]	Inhibits C5 activation and terminal complement/MAC formation	Loading 900 mg IV weekly for 4 weeks; maintenance 1,200 mg IV every 2 weeks	Total, 125 (placebo, 63; eculizumab, 62)	MG-ADL −4.2 vs −2.3 (P=0.0058); QMG −4.6 vs −1.6 (P<0.0006)	Yes
Ravulizumab	CHAMPION MG[121]	Inhibits C5 activation and terminal complement/MAC formation	Variable dosing based on weight	Total, 175 (placebo, 89; ravulizumab, 86)	MG-ADL −3.1 vs −1.4 (P<0.001); QMG −2.8 vs −0.8 (P=0.001)	Yes
Zilucoplan	RAISE[125]	Short 35 kDa macrocyclic peptide targeting C5/C5b; inhibits terminal complement/MAC formation	0.3 mg/kg BW daily SC injections	Total, 174 (placebo, 86; zilucoplan, 88)	MG-ADL −4.43 vs −2.31 (P<0.001); QMG: −6.32 vs −3.25 (P<0.001)	Yes

Iorio, 2024

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Schema di vaccinazione raccomandato nei soggetti affetti da PNH candidati a terapia con inibitori del C5 (eculizumab/ravulizumab)

- **SERIE PRIMARIA (da completarsi 2 settimane pre-terapia)**
  - Vaccino Men-ACWY: due dosi a distanza di 8 settimane
  - Vaccino Men-B: due dosi a distanza di 4 settimane

- **DOSI DI RICHIAMO (durante il trattamento)**
  - Vaccino Men-ACWY: 1 dose di richiamo ogni 5 anni
  - Vaccino MenB: 1 dose di richiamo 1 anno dopo il completamento della serie primaria, quindi 1 dose di richiamo ogni 3 anni

Girmenia C, et al Blood Rev. 2022;101013. doi:10.1016/j.blre.2022.101013

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Inibizione FcRn

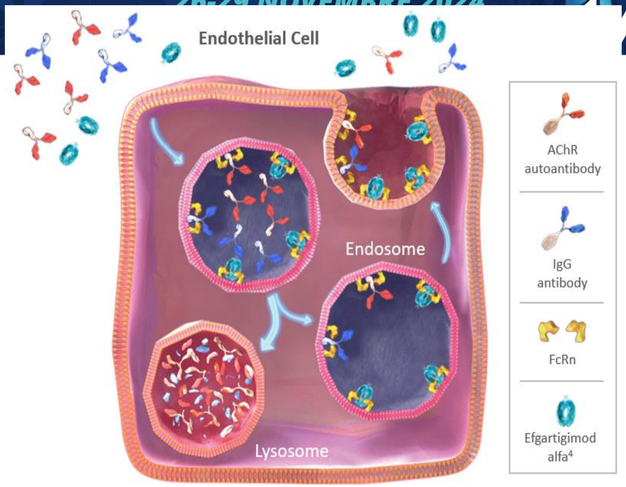

Image adapted from Kang TH, Jung ST. Boosting therapeutic potency of antibodies by taming Fc domain functions. *Exp Mol Med*. 2019;51:1–9 and distributed under the terms of the Creative Commons CC-BY license (https://creativecommons.org/licenses/by/4.0/)

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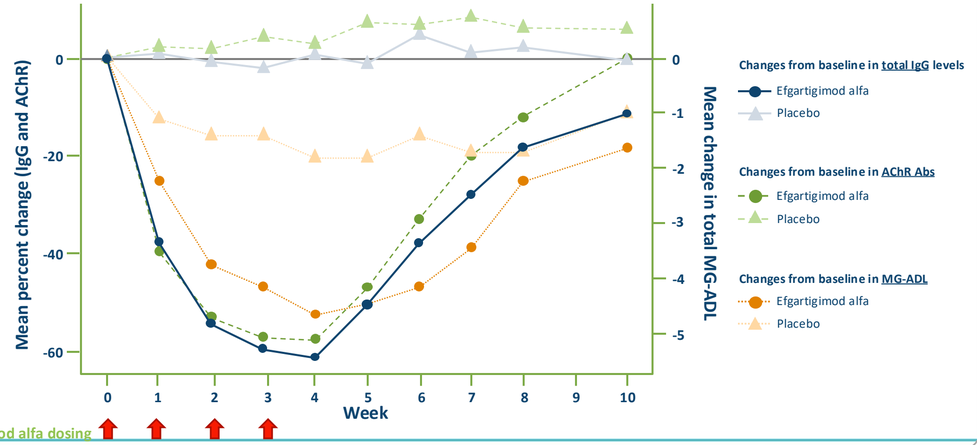
Mean percent changes from baseline in total IgG and AChR Abs levels, and total MG-ADL score



ne receptor; gMG, generalized myasthenia gravis; IgG, immunoglobulin G; MG-ADL, Myasthenia Gravis Activities of Daily Living; MoA, mechanism of action.

L, Howard JF, *et al. Lancet Neurol.* 2021;20:526–536.

MED-IT-EFG-2300047

Table 4 Summary of the neonatal Fc receptor inhibitor medications, approved and investigational clinical indications, dose, and side effects

Medication	Approved/investigational indications	Dose	Side effects
Efgartigimod IV	Approved for AChRAB+ MG in the USA, EU, JP, and CA. Still investigational for IIM and CIDP	10 mg/kg IV once a week for 4 weeks ≥ 120 kg: 1200 mg every week × 4 weeks	Headache, respiratory tract infection, and UTI
Efgartigimod SC	Approved for AChRAB+ MG in the USA, EU, and JP. Filed with US FDA for CIDP	A single SC injection (1008-mg fixed dose) over 30–90 s in cycles of once weekly injections for 4 weeks	Headache, respiratory tract infection, and UTI
Rozanolixizumab	Approved for AChRAb+ and MuSK+ positive gMG in the USA, JP, and EU Investigational for CIDP, MOG-AD, and LGI1	If weight less than 50 kg = 420 mg (3 mL). 50 kg to less than 100 kg = 560 mg (4 mL). 100 kg and above = 840 mg (6 mL). SC once weekly for 6 weeks	Headache, infections, diarrhea, pyrexia, hypersensitivity reactions, and nausea
Batoclimab	Approved for gMG in CHINA. Investigational for NMOSD	680-mg subcutaneous injections once per week for a period of 6 weeks However, both batoclimab 340-mg and 680-mg treatment groups demonstrated good results	Transient hypoalbuminemia, hypercholesterolemia, UTI, upper respiratory infection, peripheral edema. No significant headache
Nipocalimab	Still investigational for gMG and IIM	30-mg/kg loading dose, and a maintenance dose of 15 mg/kg thereafter every 2 weeks for 24 weeks [74]	gMG phase II: reversible elevations in total cholesterol LDL and HDL Reversible hypoalbuminemia, diarrhea, headache, and nasopharyngitis

AChRAb acetylcholine receptor antibodies, *CA* Canada, *CIDP* chronic inflammatory demyelinating polyradiculoneuropathy, *EU* Europe, *gMG* generalized myasthenia gravis, *HDL* high-density lipoprotein, *IIM* idiopathic inflammatory myopathies, *IV* intravenous, *JP* Japan, *LDL* low-density lipoprotein, *LGI1* leucine-rich glioma-inactivated protein 1, *MOG-AD* MOG antibody-associated disease, *MuSK* muscle-specific kinase antibodies, *NMOSD* neuromyelitis optica spectrum disorder, *SC* subcutaneous, *UTI* urinary tract infection

- Trattamento a cicli
- Possibile anche per p. AntiMuSK

CNS Drugs (2024) 38:425–441
https://doi.org/10.1007/s40263-024-01090-3
REVIEW ARTICLE

FcRn Inhibitor Therapies in Neurologic Diseases

Nouf Alfaidi[1] · Salama Karmastaji[1] · Alexandria Matic[1] · Vera Bril[1]

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https://doi.org/10.1007/s00415-024-12293-5

ORIGINAL COMMUNICATION

Real-World experience with efgartigimod in patients with myasthenia gravis

Lior Fuchs[1] · Shahar Shelly[1,2] · Ifat Vigiser[1,4] · Hadar Kolb[4] · Keren Regev[4] · Yoel Schwartzmann[1] · Adi Vakrim-Dembinsky[3,4] · Amir Dori[1,2] · Arnon Karni[1,4,5]

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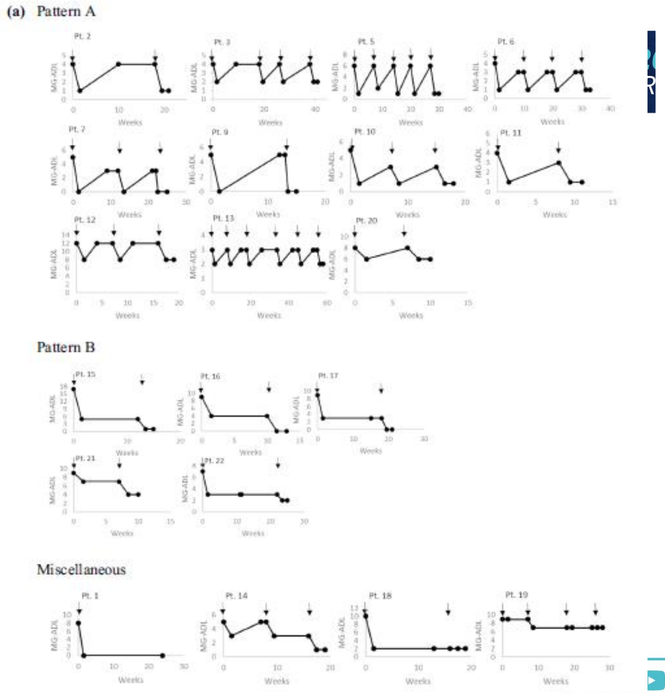

MED-IT-EFG-2300047

CRITERI RIMBORSABILITA' AIFA

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- Miastenia con anticorpi antirecettore acetilcolina
- Severità di malattia almeno di grado IIb alla MGFA
- Punteggio MG-ADL = 6 per eculizumab, ravulizumab; > o = 5 per efgartigimod
- Presenza di almeno uno tra i seguenti criteri, nonostante il trattamento standard (timectomia se indicata; corticosteroidi e almeno un altro agente immunosoppressore, utilizzati a dosaggi adeguati e per una durata adeguata) per eculizumab 2 agenti immunosoppressori
  - Almeno una crisi miastenica o evento di esacerbazione importante per anno (eventi caratterizzati da debolezza o paralisi respiratoria o bulbare, non correlati a scarsa aderenza alla terapia, infezioni o uso di farmaci che possono indurre deterioramento della MG) con necessità di ricorrere a plasmaferesi o immunoglobuline;
  - Necessità di ricorrere a plasmaferesi o immunoglobuline IGv ad intervalli regolari
  - Effetti collaterali non tollerabili / comorbidità che limitano o controindicano l'uso di immunosoppressori.

GU 5/9/2022 Eculizumab
GU 11/7/2023 Efgartigimod
GU 27/7/2024 Ravulizumab

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VIEWS & REVIEWS | OPEN ACCESS | LEVEL OF RECOMMENDATION

International Consensus Guidance for Management of Myasthenia Gravis

2020 Update

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Recommendations

Recommendation 1 is unchanged from the 2016 consensus guidance.[1]

1. Rituximab should be considered as an early therapeutic option in patients with MuSK-Ab+ MG who have an unsatisfactory response to initial immunotherapy (median 9, range 4–9).
2. The efficacy of RTX in refractory AChR-Ab+ MG is uncertain. It is an option if patients fail or do not tolerate other IS agents (median 8, range 4–9) (tables e-2 and e-7, doi:10.5061/dryad.6hdr7sqxx).

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Myasthenia Gravis Inebilizumab Trial (MINT)

This study is a phase 3, randomized, double-blind, placebo-controlled study, to be conducted at approximately 120 study sites. Approximately 230 participants (188 acetylcholine receptor antibody positive [AChR-Ab+] and 42 muscle-specific tyrosine kinase antibody positive [MuSK-Ab+]) will be enrolled…

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ISSUES & OPINIONS

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The best and worst of times in therapy development for myasthenia gravis

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CME MUSCLE&NERVE WILEY

How should newer therapeutic agents be incorporated into the treatment of patients with myasthenia gravis?

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Take home message

- ✓ **Farmaci con scarsi effetti collaterali e buona efficacia (latenza, percentuale di responder)**
- ✓ **Attualmente in pazienti con malattia non responsiva alle terapia con steroidi e immunosoppressori**
- ✓ **Meccanismo d'azione più specifico**
- ✓ **Minore effetto sul sistema immunitario di per sé**
- ✓ **Problema di farmacoeconomia e sostenibilità (100 pazienti in Toscana?)**

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