

How can patient organizations address new issues of ageing in people with rare diseases?

Myasthenia Gravis Romania

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Romanian Myasthenia Gravis Association

Forum motto:

*“Better care for rare disease patients has thankfully allowed **a longer life** for many, which has raised new questions that are discussed for the first time at Eurordis Membership Meeting.”*

- RD in adults are **NOW** considered as important issue for the RD community
- Adults with **RD** are not only “children with genetic/congenital **RD** that live longer” but some **RD** have the onset in adults or older persons (late onset **RD**)

Myasthenia Gravis autoimmune

Is the prototypical autoimmune disease in adults (Autoimmune diseases arise from an inappropriate immune response of the body against its own tissues. In other words, the immune system produces antibodies against its own cells (muscles cells))

Myasthenia Gravis = severe fluctuating muscle weakness

Epidemiology

1. Prevalence 1.4/10000
2. Female to male ratio is 1.5:1.
3. The median age at onset was 41.7 years for women and 60.3 years for men.
The clinical truism that **MG** is a “disease of young women and old men”
4. **Early onset / late onset** disease (age of 40 years was used as a dividing line between)

Tendencies

MG prevalence continuously rise

The average age of patients with MG appears to be increasing progressively.

- a) Increased survival of patients with MG
- b) Increasing of the incidence of late-onset MG

MG is part of the group of Rare Autoimmune Diseases

“TWICE RARE”

1. Rare in the general population
2. Rare inside the RD group (majority are genetic/congenital RD)

Resulting in:

- * Autoimmune RD are marginalized inside the RD group (“democracy is the dictatorship of the majority”)
- * Low interest for research, national programmes, drug companies
- * Low interest from mass media
- * Low visibility in the public awareness campaign for fundraising (people give money for children because of higher emotional impact of media campaigns)
- * Low represented inside Eurordis - difficult to make our voice heard



Who am I?

WHO are we?

Public awareness
campaign by patients
associations

MG may affect anyone, anytime



I am Male



I am Female



I am any age



I am any race



I am any colour

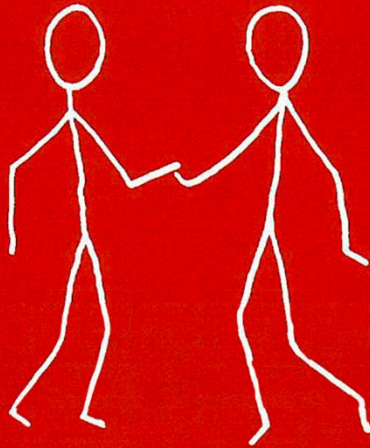
Please
don't
let
me
down



I may never get up!
Today you may not care. . .
Tomorrow YOU may be
cared for!

Very variable, fluctuating symptoms

Yesterday. . .



I was
as strong
as you

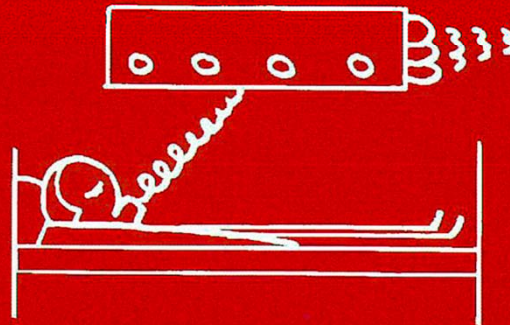
Tomorrow when I
wake up. . . I may be as
strong as you, or. . .



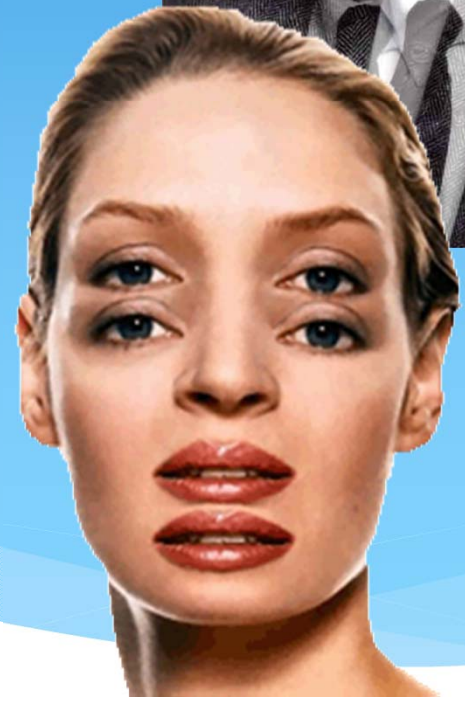
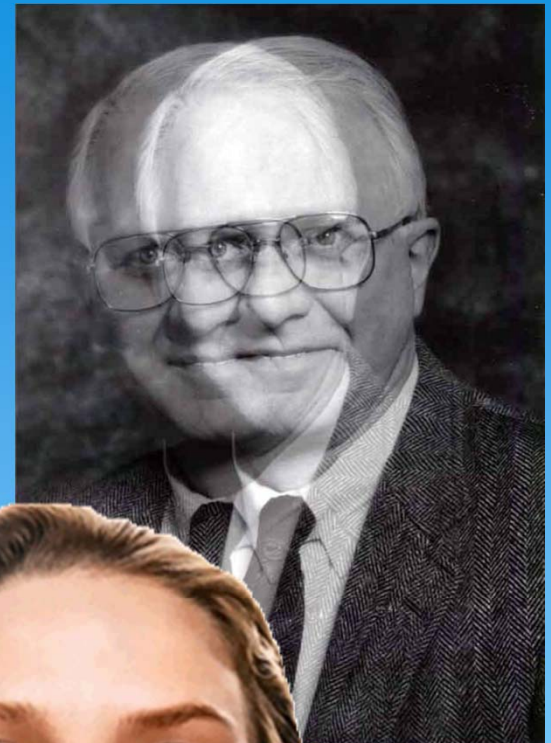
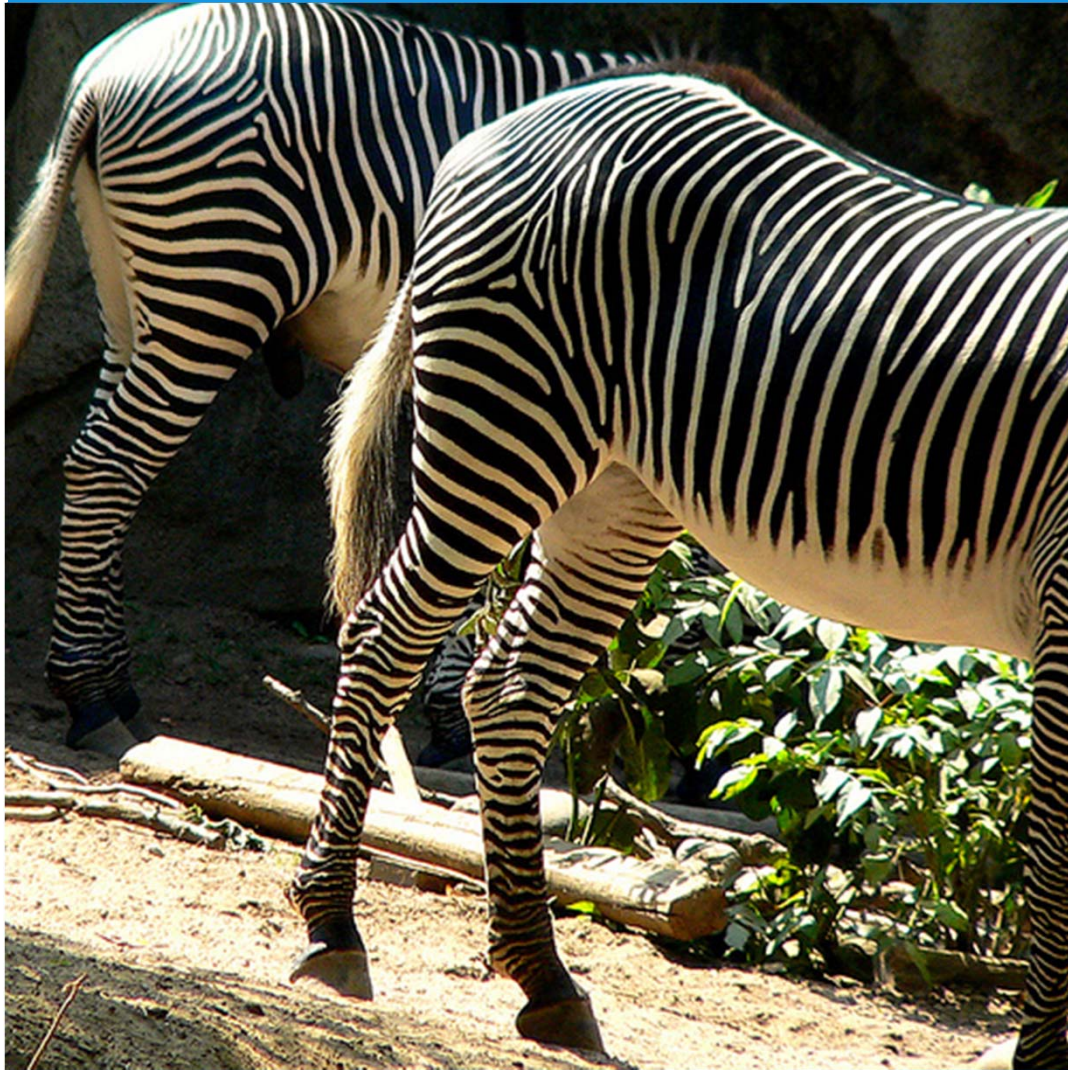
I may fall
down and
be
mistaken
for a drunk
or an
addict.

Myasthenia Gravis = Medical emergency!

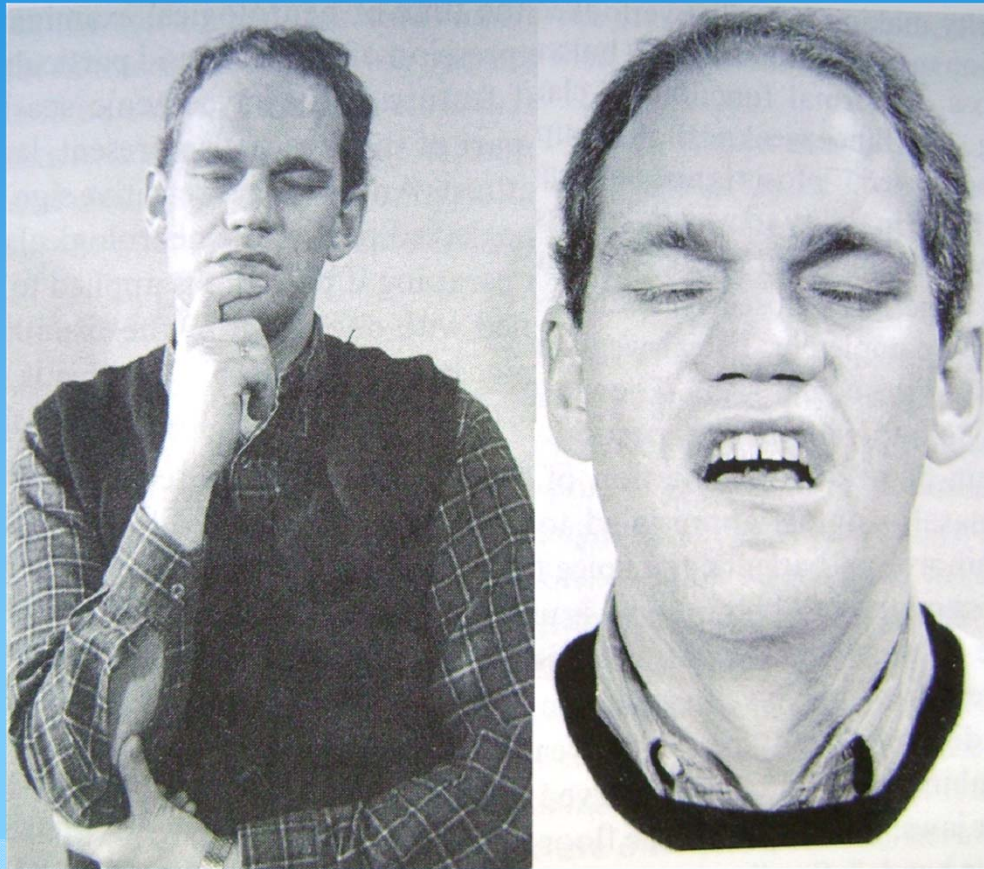
Today I cannot move...
a finger
a toe
or breathe



without the aid
of a respirator



MG symptoms: double vision



MG symptoms:
ptosis of eyelids, facial weakness (jaw support)





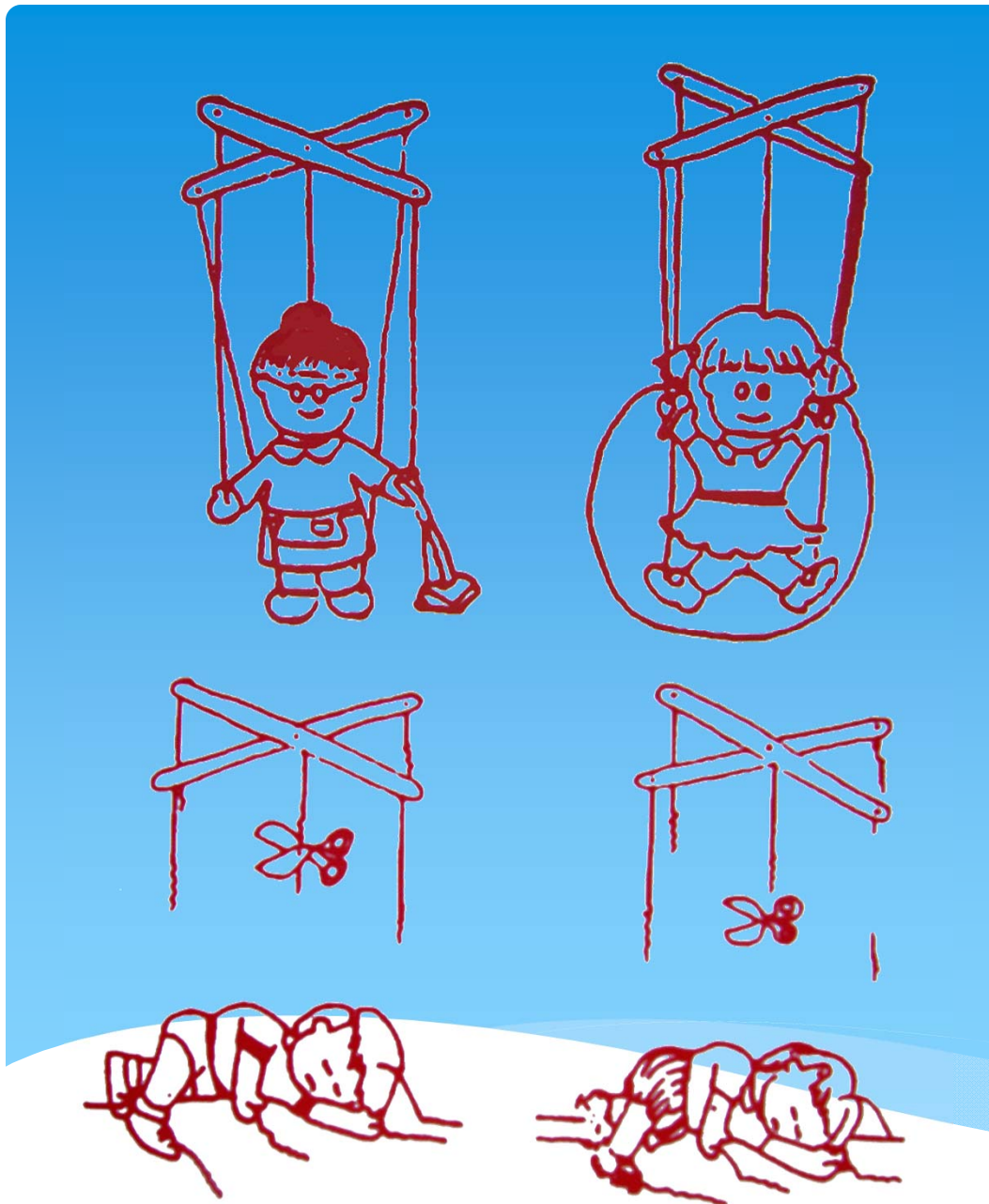






“The rag doll illness”

A Registered Charity 1046443



Quality of Life Ratings for MG Patients compared with Health Control Subjects

From Henry J. Kaminski MD, Myasthenia Gravis and related disorders (2nd edition 2009)

SF-36 scale	MG patients	Health controls
Physical Functioning	52.7	84.5
Social Functioning	72.2	83.6
Role Disruption-Physical	25.9	81.2
Role Disruption-Emotional	70.3	81.3
Mental Health	74.2	74.8
Vitality	45.1	61.1
Bodily Pain	70.5	75.5
General Health	56.4	72.2
Overall quality of life	58.4	73.0

Range = 0-100; higher scores reflect better function.

The role of the patients organization

1. Identify problems from calls on help lines (caller profile analysis)
support groups, forums, networks
most important issues = diagnosis and treatment

2. Information and education

- a) informative materials addressed directly to the patients
(not to parents or caregivers)
- b) providing psychological /emotional support is crucial
- c) education for an “expert patient “
 - education for a healthy live(diet, physical activities)
 - complications prevention, avoid crises,
 - home injuries prevention

MG in adults - Diagnostic issues

- **Difficult and often delayed (years)**- because of invisible, variable disease
- **Confusion** with other common chronic pathologies added with ageing (cardiovascular diseases, diabetes, rheumatic degenerative), (diagnostic pitfalls)
 - MG with early onset is mistaken for ocular, neurological, endocrinology or psychic disorders
 - MG late onset mistaken for stroke
 - Specific issues due to physiological conditions (puberty, pregnancy, menopause)
- MG associated with other/new autoimmune diseases (Sjogren's syndrome, lupus thyroiditis, Behcet, rheumatoid arthritis, pemphigus etc.)
(Patients may have 2-3 or more autoimmune diseases)

.

MG in adults - Treatment issues

Medication should be personalized

Each patient is unique, we are called “snowflakes “(all the same, each one unique)

Aware of medication that aggravated MG

(flyer provided by patients associations)

- Drugs combinations

- with medications for other chronic diseases
- with treatment of specific drug adverse reactions (corticoids, immunosuppressive therapy) like osteoporosis, ulcer, gastritis, diabetes, hypertension, glaucoma, etc.)

- Specific treatment issues:

- Surgery, anesthesia, dental surgery
- Pregnancy, delivery, breast feeding
- Immunizations



Lista medicamentelor care pot agrava Miastenia Gravis

Material informativ cu scop educațional

List of drugs that may aggravate MG

ATENȚIE!

Pentru medicii care au în tratament
pacienți cu Miastenia gravis

Medicamente interzise în Miastenia gravis

- A) D-penicilamina nu trebuie utilizată
NICIODATĂ la pacienți cu miastenie*
- B) Urmatoarele medicamente produc agravarea
miasteniei la majoritatea pacienților și trebuie
evitate sau utilizate cu prudență și pacientul să
fie atent monitorizat:*
- 1) succinilcolina, d-tubocurarina,
vercuronium
și alți agenți blocați neuromusculari
 - 2) chinina, chinidina și procainamida
 - 3) unele antibiotice, în special: tobramicina,
gentamicina, kanamicina, neomicina,
streptomycină, colistin, eritromicina,
ciprofloxacina, telitromicina
 - 4) betablocanțele: propranolol, timolol
maleat picături pentru ochi
 - 5) blocanțele canalelor de calciu
 - 6) substanțele de contrast iodate
 - 7) magneziul și preparatele ce conțin
magneziu
 - 8) Interferonul alfa și beta
 - 9) Toxina Botulinică (Botox)

Agravarea slăbiciunii la pacienții miastenici a fost raportată și la alte medicamente. Toți pacienții cu Miastenia gravis trebuie urmăriți ori de câte ori se instituie o nouă medicație.

Lista cu medicamentele ce pot agrava Miastenia Gravis

I. Medicamente care pot exacerba miastenia
sau evidenția o miastenie latentă

- Blocante neuromusculare:
(curarizante, ex Vercuronium®)
- Excesul de anticolinesetrazice:
pyridostigmina (Mestinon®)
sau neostigmina (Miostin®)
- Corticosteroidii și ACTH (Prednison, uneori la
începutul tratamentului)
- Magneziu și preparatele ce conțin magneziu
- Antiaritmice:
 - Lidocaina (Xilina) administrată intravenos
(local se poate administra fără riscuri)
 - Chinidina și preparatele pe baza de chinina
 - Procainamida (Pronestyl®)
- Fenitonia și medicamentele înrudite (ex. Dilantin®)
- Antibiotice
 - aminoglicozide (gentamicina, tobramicina,
amikacina, neomicina, streptomycină,
kanamicina)
 - polipeptide: polimixina, colimicina (Colistin)
 - miscelane (alte antibiotice) clindamicina,
ciprofloxacina, eritromicina, telitromicina
(KETEK®)

II. Medicamente ce pot induce miastenia
prin provocarea unei reacții autoimune

D-penicilamina (Cuprimine®)
Trimetadiona (Tridione®)

III. Medicamente implicate în agravarea
miasteniei în cazuri izolate

Cimetidina (Tagamet®)
Anestezice (inclusiv alcoolul)
Chloroquina
Cocaina
Diazepam (Valium®)
Lithium
Propranolol
Timolol maleat (Timoptic®) - picături
pentru ochi
Tetracilinele:

- tetraciclina,
chlortetraciclina,
oxytetraciclina,
demeclocyclina,
doxycyclina (Vibramycin®),
minocyclina

Trihexyphenidyl (Artane®)

Important pentru pacienți:

**Evitați automedicația!
Consultați specialistul neurolog.**

Adaugarea unui nou medicament poate produce agravarea miasteniei, mai ales daca pacientul se afla intr-un puseu evolutiv. Medicamentele din lista de mai jos sunt cele care produc cel mai frecvent probleme. Orice nou tratament si riscurile acestuia trebuie discutate cu medical dvs, curant.



Antibiotice

Colistin
Kanamicina
Streptomycină
Tetraciclină
Telitromicina (Ketek)

Cardiovasculare

Chinidina
Procainamida
Propranolol

Hormoni

Corticosteroizi
Tiroxina

Anticonvulsivante

Fenitoina
Penicilamina

Psihotrope

Litiu
Promazina

Altele

Magneziu
Laxativele

Anestezice

Relaxante musculare

Acest card este emis de :
Asociația Națională Miastenia Gravis
Romania
Str Gral Macarovici 7
sector 6 Bucuresti, 060142
Romania
e-mail: asociatia.miastenia@gmail.com
www.miastenie.ro
Tel. 0744.704.399
0743.470.122

ATENȚIE

ÎN CAZ DE URGENȚĂ

Aceasta persoana suferă de

MIASTENIA GRAVIS

Medication information card



Nume Rădulescu

Nadia

Adresa Strada General Macarovici
George, nr 7, sect.6, București

Telefon 0744.704.399

Data nașterii 24 septembrie 1957

Aceasta persoană suferă de

Miastenia gravis [X]

Sindrom Lambert Eaton []

Sindrom miastenic congenital []

În prezent este sub tratament cu:

Medicament	Dozaj
Medrol	6mg/zi (ora 6:00)
Mestinon	30 mg de 6 ori/zi (6-10-13-16-19-23)

Dozajul si timpul de administrare sunt foarte importante si este recomandat sa nu fie modificate.

ÎN CAZ DE URGENȚĂ

Contactați:

Medicul de familie

Nume: Sângeorzan Ancuța Michaela

Contact: 0745. 599.215

Medicul curant:

Nume: Vâlcu Crisanda

Clinica: Neurologie Fundeni

Contact: 0723. 519.223

Persoana apropiată:

Nume: Romeo Begheș

Contact: 0754. 929.120

Primul ajutor în caz de înecare cu alimente Manevra Heimlich



Primul ajutor în caz de înecare cu alimente, când persoana se învinețește și nu mai are aer.

A. Manevra Heimlich

Salvatorul se așează în spatele persoanei, cu pumnul așezat în zona sternului, apasă brusc și tare strângând mâinile în jurul mijlocului, ridicând-o brusc în sus, până elimină restul de mâncare.

B. Singur acasă

În cazul în care ești singur acasă și te-ai înecat, poți să ieși aceste măsuri de prim ajutor și singur. Apleacă-te peste un scaun, apăsând brusc pe zona abdomenului. În toate cazurile, sunați la 112.

Miastenia Gravis este o boală autoimună neuromusculară caracterizată prin slăbiciune fluctuantă a unor grupe de mușchi voluntari, cel mai frecvent afectați fiind mușchii faciali, ai masticăției și deglutiției, ai centurilor scapulară și pelvină, precum și mușchii respiratori.

Semnele și simptomele cele mai comune sunt căderea pleoapelor, vedere dublă, vorbire neclară sau imposibilitatea de a vorbi, regurgitarea lichidelor pe nas, tuse slabă, probleme la mestecat și înghițit, dificultate în a sta și a se ridica de pe scaun, de a ține capul ridicat, probleme la mers, sufocare, respirație dificilă.

Urgențele miastenice ("Crizele") sunt rare, dar pot surveni când mușchii respiratori devin foarte slăbiți încât respirația devine foarte dificilă, superficială și ineficientă. Căile respiratorii se pot obstrua din cauza slăbiciunii musculaturii orofaringiene și acumulării de secreții.

DACĂ ACESTE SIMPTOME NU SUNT TRATATE PROMPT SE POATE AJUNGE LA AFECTAREA SCHIMBULUI DE GAZE ȘI LA INSUFICIENȚĂ RESPIRATORIE SAU STOP RESPIRATOR

Asociația Națională Miastenia Gravis România

Str. G-ral. George Macarovici nr. 7, sector 6, cod 060142,
București, România

Tel: 0744704399; 0743470122; 0314013830;

Fax: 021.7603023

Email: asociatia.miastenia@gmail.com

Website: www.miastenie.ro

CIF: 18136030; Nr. înreg. Registru special: 70/08.11.2005

IBAN: RO55BPOS71906938789RON01,

BancPost București - Agenția Lizeanu

ALERTE



PRIMUL AJUTOR DE URGENȚĂ ÎN MIASTENIA GRAVIS

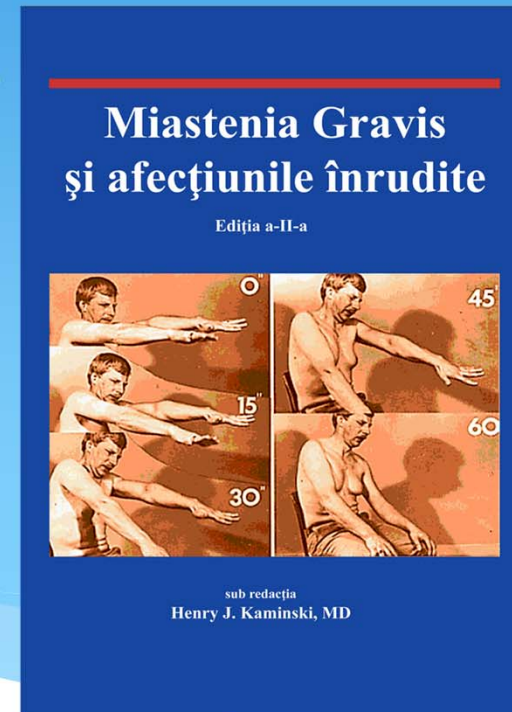
**Informații importante
pentru pacienți, anturaj,
paramedici, personal
medical UPU**

The National MG INFO CENTRE

INFO MG-RO

We provide **literature** on the disorder and treatment, available through the national office and the website (www.miastenie.ro).

We translated, published and disseminate the book:
Myasthenia Gravis and related disorders by **Henry J Kaminski MD**,
project founded and realized 100% by our association's contribution.



INFO MG-RO

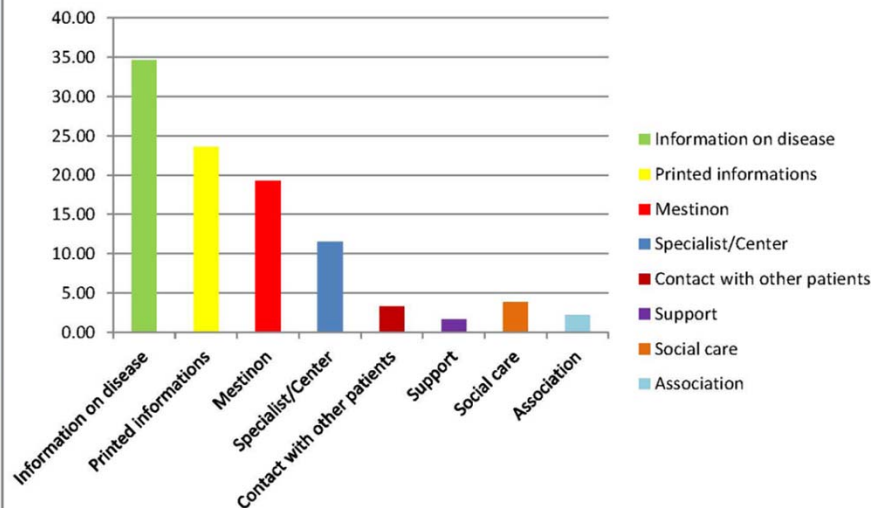
Member of the European Helplines Network (Eurordis)

Caller profile analysis

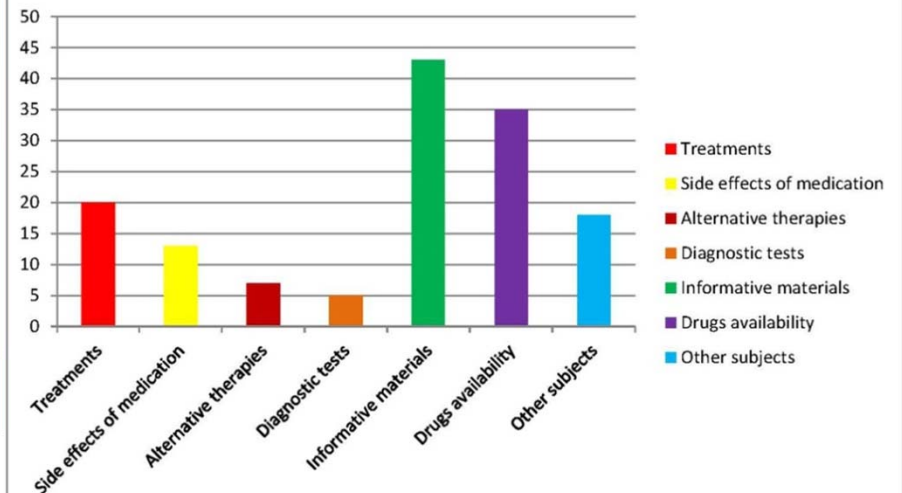
The only EU helpline focused on a single rare disease

INFO MG-RO offers information on the disease, medical centers and specialists, local patients support groups and contact with other patients, about social care, medication availability, psychological support.

Purpose of call



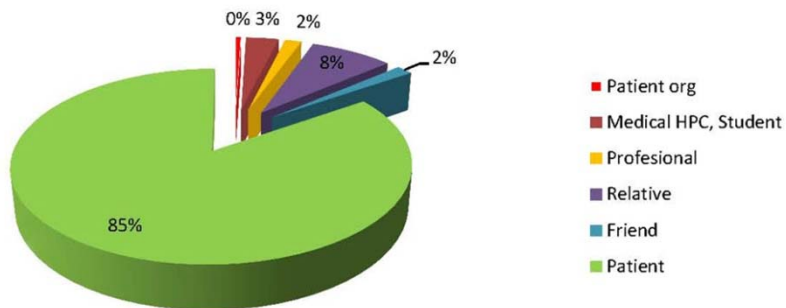
Information on disease



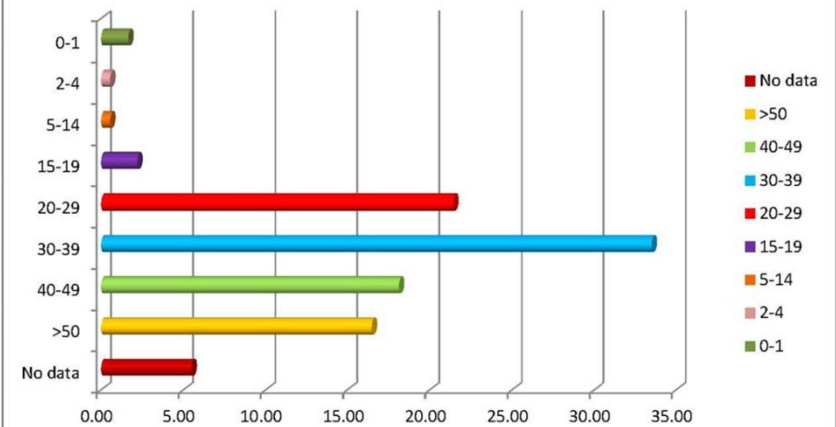
Caller profile analysis

Based on 182 calls in 8 months

Category of caller



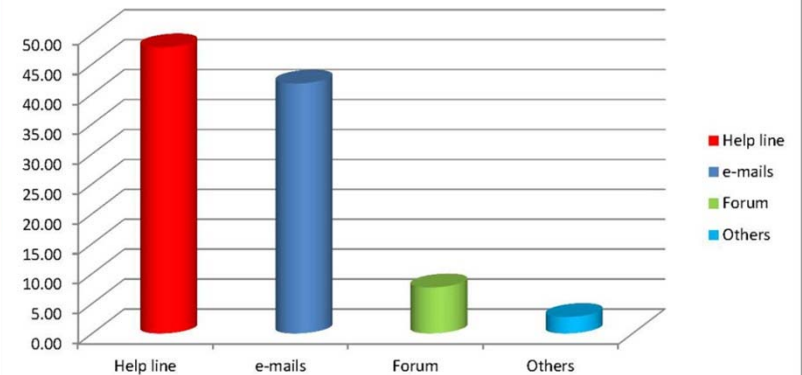
Age groups %



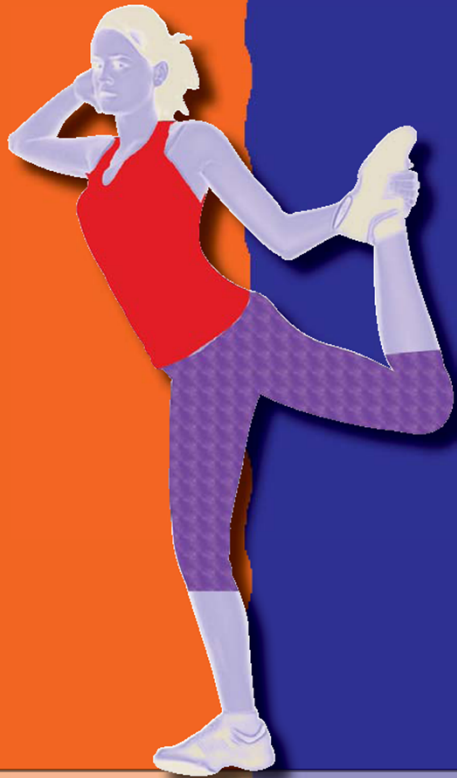
Gender



Type of contact



Josipa Gazibara



**Program de
kinetoterapie pentru
persoanele cu
miastenia gravis**

Ediția a II a

Rehabilitation programmes

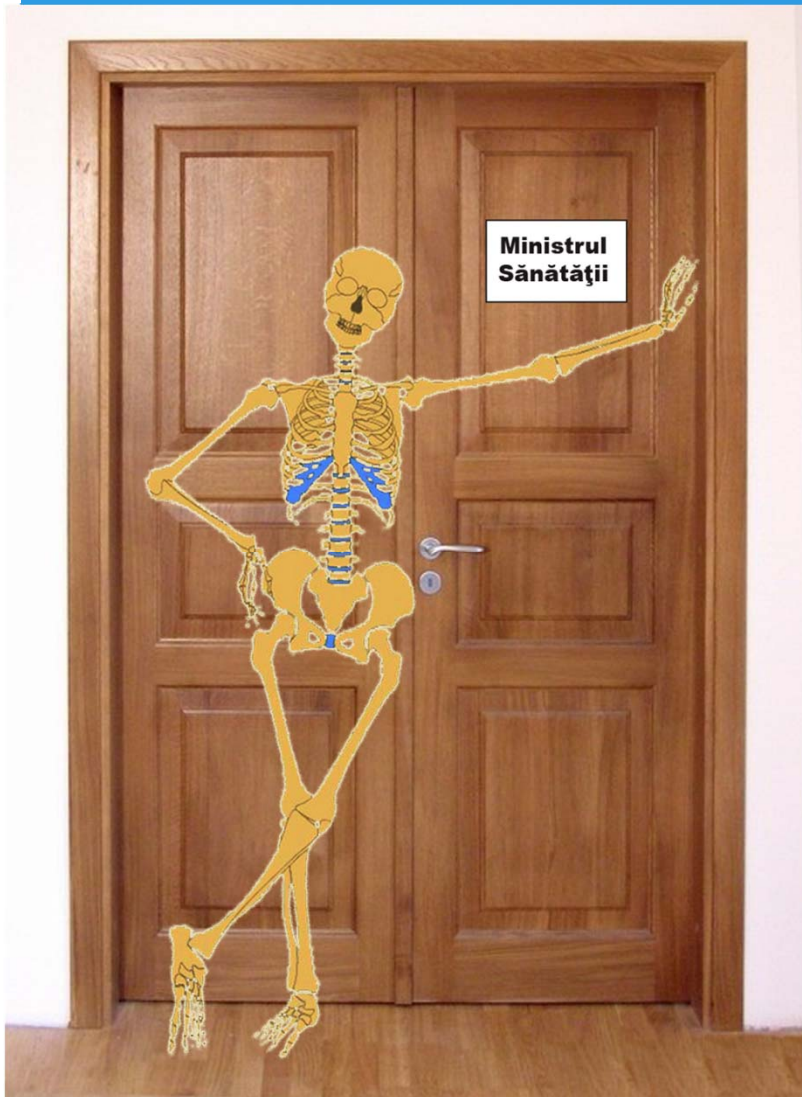
Living with MG as an adult

- Adults with MG are young active peoples, the majority have a job, an active social life
- Have to cope with a disabling disease
- Adapt physical activities

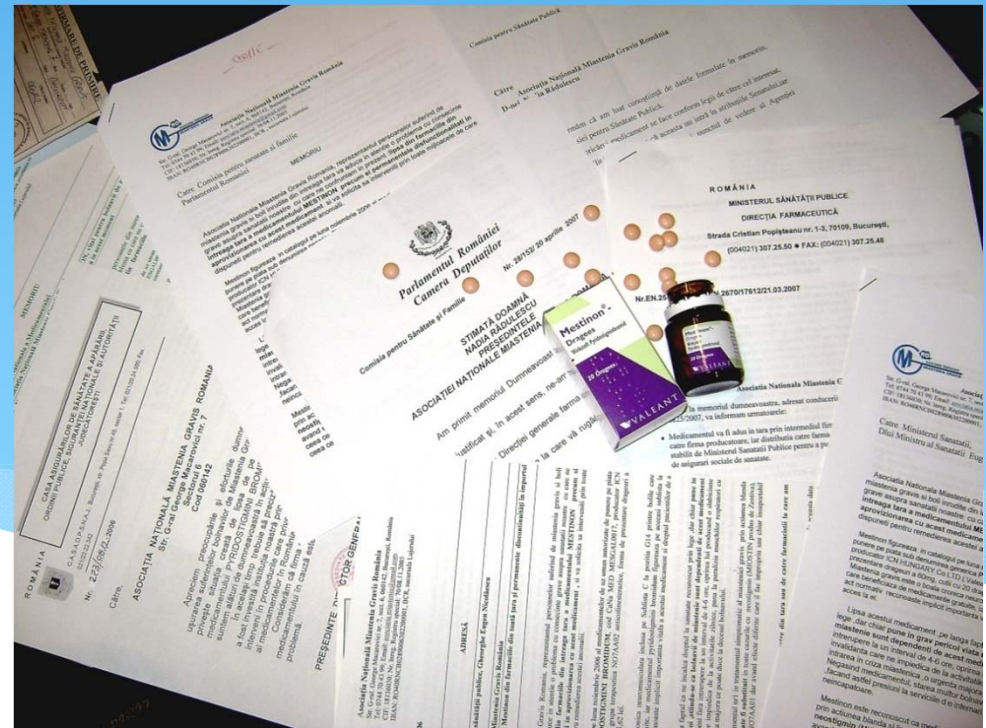
Public campaign "Myasthenia Gravis - The invisible disease"



Public campaign : Mestionon crisis in Romania Fighting against bureaucracy



Memoires to Health Minister, Parliament, Drug Agency, Lobby Parliament, European organizations.



Campaign: "Patient in the center of health system"



Legislation



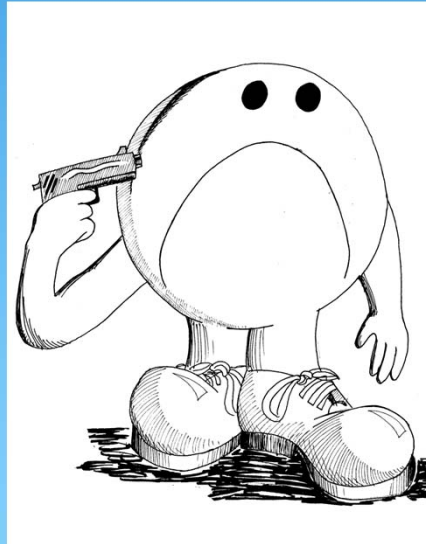
Minister



General practitioner

National Drug Agency

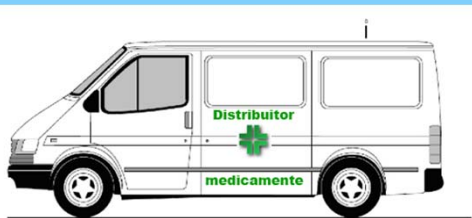
Patient



Specialist



Pharmacist



Distributor

MG Patients Associations

Collaboration with other patients organizations focused on:

- fundraising
- patients rights, human rights

Alliances -European : EuMGA, EURORDIS

-National : Romanian Alliance for RD

Alliance of patients with autoimmune disorders

Participation to clinical trials epidemiological studies, databases :

- Extension of the existing databases
- Characterization of the general criteria of MG population
- Characterization of the diversity of MG patients forms
- Determination of the influence of several factors on MG incidence

European Myasthenia Gravis Association

Third Annual Meeting and
General Assembly
May 26, 2012, 9:00am
MCE Conference Centre Brussels
Room 4E, 4th floor

 EuMGA

Under the
patronage of



EURORDIS
Rare Diseases Europe

