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Genetic Neuromuscular Diseases in Libya.

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Letter to Editor

Dear Editor:

Whereas in previous decades the landscape for certain disorders such as cancer and genetic diseases, in particular neuromuscular, was bleak, a glimmer of hope has now arisen. This glimmer is growing fast into a beam that promises to shine light over the coming decades. Genetic therapy has finally arrived.

I wish to shed light on the prospects in our location here in Libya: what has been done so far and what remains to be done. Our experience over the last 4 years will also be outlined. The reader will find that we have much to be proud of, and yet the challenges have been sometimes seemingly insurmountable.

The scientific committee for genetic neuromuscular diseases (NMDs) was instituted in Libya four years ago. (1) Members include adult and pediatric neurologists' representatives from most of the main cities of Libya as well as legal, pharmacist, and administrative members. It is part of the Libyan Program for Neuromuscular Diseases, which includes the Patient Neuromuscular Society. Thus, Libya has made considerable progress in the promotion of patient advocacy for these conditions. The neurologist members of the scientific committee are the heads of subcommittees formed in the city or city location to which they belong. Thus, a network covering all of Libya has been formed. The main concept overriding this organization is teamwork. Patients attend the subcommittee clinics to be assessed, tested genetically, counselled, treated, and followed up. Accurate statistics can be obtained regarding these diseases' incidence and prevalence in Libya. A national patient database is now the next aim.

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GENETIC TESTING

Around 1052 patients had genetic testing, and from the results we found spinal muscle atrophy (SMA), Duchenne muscle dystrophy (DMD), and other NMDs, with limb girdle muscle dystrophy being the most prevalent (Figure 1). (1)

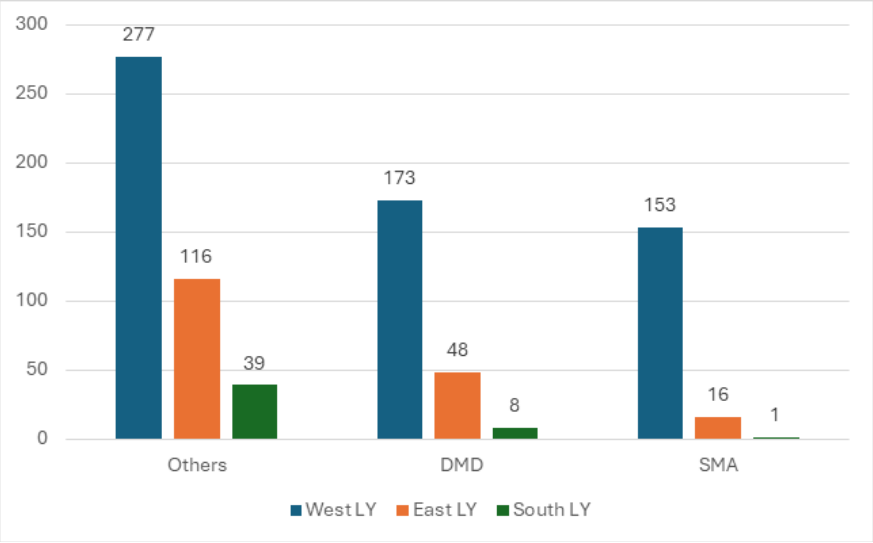


Figure 1: Genetic neuromuscular diseases in Libya 2022-2024.

Libya's Epidemiology for Genetic Neuromuscular Diseases:

We calculated the prevalence of SMA and DMD as 3.02/100,000 population and 7.06/100,000 males, respectively. (1)

With regard to SMA in particular, this was considered to be high as compared to the rest of the world and may be the result of consanguineous marriages in our location for this autosomal recessive disorder. This highlights the importance of prenatal genetic testing, neonatal screening, and family counselling and planning, especially in families with a history of genetic diseases. It is our hope to participate in a national program and campaign for genetic diseases, which would address all these aspects of diagnosis, prevention, and treatment where possible.

GENETIC THERAPIES:

The field of treatments for various genetic diseases is rapidly expanding; the mechanistic approaches include those for SMA. Nusinersen is an antisense oligonucleotide given intrathecally, and Risdiplam is a small DNA splicing protein that is given orally and is now freely available for SMA patients in Libya (around 150 patients). Gene therapy with Onasemnogene abeparvovec is given early to patients before age 2 years who weigh less than 21 kg and corrects the gene defect. An adenovirus vector carries a new gene into the patient's DNA to form the missing SMN protein. (2) It is a highly specialized treatment for which so far Libyan children have been sent to Egypt (18 patients) and, following that, to the United Arab Emirates (6 patients so far), where they received the therapy at specialized centers for genetic diseases. The treatment is now available to be given intrathecally for ages above 2 as well and higher weights. (3) The challenge for most health services will be meeting the cost of the expensive treatments.

Genetic therapies for Duchenne Muscle Dystrophies: include exon-skipping therapies with antisense oligonucleotides; 103 patients with appropriate gene mutations received their first doses of these intravenous therapies. (4) Ataluren was given to 40 patients with nonsense mutations but has been

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stopped pending FDA decision, as in a study it was found not to be sufficiently effective. (5) Luckily, givinostat, a histone deacetylase inhibitor, which has also been used in hematological conditions, (6) will soon become available for all patients with DMD who do not receive exon-skipping medications, around 200 patients. (7) Steroids are provided to all patients.

The plan is now to make gene therapy delandistrogene moxeparvovec-rokl available inside Libya for mobile patients with DMD. This therapy is with an adenovirus vector carrying a micro-dystrophin gene, which will be incorporated in the patient's DNA to form a functioning micro-dystrophin protein. It is approved for ambulatory individuals at least 4 years old with Duchenne muscular dystrophy (DMD) who have a confirmed mutation in the DMD gene. (8) The therapy will require intensive care and other specialized facilities as well as training of staff. We aim to collaborate with teams in the region who have experience in giving this therapy with success.

Vaccinations and Vaccination Campaigns:

The Scientific Committee provides guidelines and supervises programs that aim to cover all aspects of the genetic neuromuscular patients' daily life. Recently a national influenza vaccination program was organized.

The Multidisciplinary Team Concept:

This concept, although new in our health service, was readily adopted by the Scientific Committee, and although the challenge was and remains to provide such multiple specialty teams to all the major cities in Libya, the fact that treating physicians are now aware that patients must be approached holistically has been a great advancement in their care.

Social coverage and patient advocacy:

The plight of patients with neuromuscular disease in a developing country environment clearly reached all concerned, including the authorities and the Libyan public. A very close relationship exists between the patients, their families, and the Scientific Committee through the Libyan Neuromuscular Patient Group, which is a main stakeholder in the Libyan Program for Genetic Neuromuscular Diseases. Patients in Libya voice their concerns and state their individual as well as community requirements loudly, and they are heard. The Scientific Committee liaises with the government-funded departments concerned, including the Social Security Services, to ensure their needs are met. This is aimed at providing where needed BiPAP machines, cough assist devices, and electronic wheelchairs, as well as home adaptations and other necessities for the daily life of these patients.

Physiotherapy and rehabilitation:

The challenge was to provide specialized physiotherapy and rehabilitation services to all patients with these conditions all over Libya. Therefore, several training courses were arranged for physiotherapists locally and abroad, as well as a resident visiting specialist team from Slovakia. The visiting team provides both treatment for patients and training of local physiotherapists.

Surgery for Scoliosis: It is recognized that scoliosis is a debilitating complication of neuromuscular diseases, which, having been brought to the attention of our authorities, led to several visits by specialized surgical teams to perform corrective surgery in Libya and train Libyan surgeons to perform this surgery in the future. This surgery is performed under neurophysiological and radiological control, and therefore training in these specialties is also required.

Genetic treatment in Libya: The Future: The future aims to keep Libya in the mainstream of the rapidly advancing field of genetic discoveries, whether in testing and diagnosis or the development of effective new therapies. Adenovirus vector gene replacement is not the only mechanistic approach. CRISPR-Cas9-based therapies are also being considered and are in the pipeline. (9) We are likely to get therapies for other forms of genetic neuromuscular diseases such as limb girdle muscle dystrophies. In order to keep up, Libya must upgrade the health service, train specialists in genetics, and provide genetic laboratories and newborn screening, as well as genetic clinics where counselling can be given. We must also provide and equip multidisciplinary teams working in specialized centers and also promote and

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fund research.

CONCLUSION:

Libya is now considered a model state in North Africa for addressing the needs of patients with genetic neuromuscular diseases and funding genetic therapies for its patients. We have come a long way since these diseases were considered hopeless for patients, but there is still much more to be done.

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