Development of Novel Muscle-Active Agents: A Regulatory Perspective

Author: *Ewa Bałkowiec-Iskra[1]

1. Medical University of Warsaw, Poland
*Correspondence to ewa.balkowiec-iskra@wum.edu.pl

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BACKGROUND AND AIMS

Sarcopenia is a severe and debilitating disease, affecting muscles and bones, as well as other organs and systems. Sarcopenia is characterised by progressive loss of skeletal muscle mass, strength, and function. It is estimated that sarcopenia affects approximately 10–40% of adults aged 60 years and older, with prevalence rates increasing in subjects over 80 years. The clinical implications of sarcopenia include, but are not limited to, increased risk of falls, fractures, functional disability, and mortality, leading to healthcare burden and reduced quality of life. Sarcopenia is a significant public health concern in the context of global population ageing.[1,2]

DISCUSSION

The European Working Group on Sarcopenia in Older People 2 (EWGSOP2) has established comprehensive diagnostic criteria, incorporating measures of muscle strength, mass, and physical performance. However, sarcopenia is frequently overlooked in clinical practice. Many patients with sarcopenia are not seen by geriatricians, but by general practitioners or other specialists who may not be trained to assess muscle loss, weakness, and functional loss.[3] The underlying pathophysiology of sarcopenia remains poorly understood and is complex and multifactorial, involving age-related alterations in protein synthesis, inflammatory pathways, hormonal changes, and mitochondrial dysfunction.[4]

Pharmacological interventions that have been studied in sarcopenia include androgenic medications (testosterone), selective androgen receptor modulators, growth hormone, myostatin inhibitors (antimyostatin antibodies and myostatin propeptides), angiostatin-converting enzyme inhibitors, β2-adrenergic receptor agonists (formoterol), growth differentiation factor 15 monoclonal antibodies, and antidiabetic medications (glucagon-like peptide-1 receptor agonists and sodium-glucose co-transporter 2 inhibitors). Moreover, enhancement of mitochondrial function, improvement of insulin sensitivity, and regulation of muscle energy metabolism are considered possible pharmacological interventions that could be effective in patients with sarcopenia.[5] Designing clinical studies in sarcopenia is very challenging due to heterogeneity of the population and a lack of any regulatory guidelines. Open questions include, but are not limited to: selection of the target population (inclusion criteria, exclusion criteria, comorbidities), outcomes (primary and secondary efficacy and safety endpoints), overall study design (dose selection, comparator, duration, biomarkers, non-pharmacological interventions, statistical analysis, minimally important clinical difference), demonstration of a maintenance of effect, and prevention of relapses.

CONCLUSION

The European Medicines Agency (EMA) offers multiple tools supporting the development of medicinal products (scientific advice and protocol assistance,

qualification advice, Priority Medicines [PRIME] Scheme).[6] More constructive interactions involving different stakeholders (researchers, industry, regulators) are needed to discuss various aspects of study design.

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