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DIAGNOSIS AND TREATMENT OF CONGENITAL HEMOPHILIA WITH INHIBITORS A LATIN AMERICAN PERSPECTIVE

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Summary The Committee of Latin America on the Therapeutics of Inhibitor Groups (CLOTTING) is composed of a number of hemophilia specialists from Latin America. The group aims to encourage the adoption of a good standard of care for Latin American patients with hemophilia. The occurrence of inhibitors in patients with hemophilia poses clinical challenges, and it is estimated that between 1 000 and 3 000 patients in Latin America are affected by hemophilia with inhibitors. There is an urgent need to establish a regional consensus and clinical guidelines for the diagnosis and treatment of these patients. We present an extensive review based on best current clinical practice and published literature, as seen from a Latin American perspective, taking into account the variable nature of hemophilia care available in the various countries in this Region.

Key words: hemophilia, inhibitors, treatment, hemophilia guidelines

Resumen ***Diagnóstico y tratamiento de la hemofilia congénita con inhibidores. Una perspectiva latinoamericana***. El Comité Latinoamericano sobre la Terapéutica de Personas con Inhibidores (CLOTTING) está compuesto por un grupo de especialistas en hemofilia de Latinoamérica. El objetivo del grupo es promover la adopción de un estándar de tratamiento óptimo para los pacientes con hemofilia en Latinoamérica. La prevalencia de inhibidores en pacientes con hemofilia en Latinoamérica determina desafíos clínicos y se estima que de 1 000 a 3 000 pacientes en esta región están afectados con hemofilia e inhibidores. Existe una necesidad urgente de establecer un consenso regional y guías clínicas para el diagnóstico y tratamiento de estos pacientes. Nosotros presentamos una revisión exhaustiva basada en las mejores prácticas clínicas vigentes y en los datos publicados en la literatura, con una perspectiva latinoamericana, tomando en cuenta la variabilidad existente de los tratamientos de la hemofilia disponibles en los diferentes países de esta Región.

Palabras clave: hemofilia, inhibidores, tratamiento, guías clínicas

Hemophilia is an inherited bleeding disorder characterised by frequent bleeding episodes, particularly affecting joints and muscles. Treatment with clotting factors can control the bleeding, preventing chronic arthropathy and premature death. However, in as many as 20-30% of patients with hemophilia A (who have a deficiency of factor VIII [FVIII]), and 3% of patients with hemophilia B (who have a deficiency of factor IX [FIX]), replacement therapy with coagulation factors is associated with the development of inhibitors[1, 2].

Inhibitors are neutralising antibodies that interfere with the function of FVIII or FIX. The occurrence of inhibitors has significant clinical implications (the response to treatment becomes uncertain, morbidity is increased and life expectancy is reduced). In addition, direct medical costs are much higher for hemophilia patients who have inhibitors, as are the non-medical costs to the patients, their families and society[3].

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