

Complementary information to the article: Recommendations for management and follow-up in patients with transthyretin-mediated hereditary amyloidosis

Información complementaria: recomendaciones de manejo y seguimiento en pacientes con amiloidosis hereditaria mediada por transtiretina

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Bogotá, D.C., February 2, 2024

Dear Editor:

The following complements the declaration of conflicts of interest and provides some clarifications for the article titled “Recommendations for treatment and follow-up in patients with transthyretin-mediated hereditary amyloidosis,” published in *Revista Colombiana de Cardiología*, Volume 30, No. 6, 2023.

After reading the questions, the first thing we noted was that the document includes diagnostic and follow-up elements in patients with transthyretin-mediated amyloidosis and we did not consider any recommendations for specific treatment. Thus, the title that best reflects the content is “Recommendations for diagnosis and follow-up of patients with transthyretin (TTR)-mediated hereditary amyloidosis.”

The recommendations we have given for follow-up are (in order):

- The role of genetics in hereditary transthyretin-mediated (hATTR) amyloidosis.
- Neurological follow-up in symptomatic patients.
- Complementary tests in patients with hATTR and carpal tunnel syndrome.
- Complementary autonomic nervous system assessments in patients with hATTR.
- Cardiology assessment in patients with hATTR.
- Nephrological follow-up in patients with hATTR.
- Ophthalmological treatment of patients with hATTR (in which ophthalmologists recommend symptomatic treatment).

This working group began as an academic initiative among colleagues. We are a group of physicians from

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Date of reception: 15-03-2024

Date of acceptance: 21-03-2024

DOI: 10.24875/RCCARE.M24000089

Available online: 13-05-2024

Rev Colomb Cardiol. 2024;31(2):118-120

www.rccardiologia.com

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different specialties who began to diagnose patients with TTR amyloidosis in Colombia. As we needed the intervention of other specialties, we established an interest group that met at various gatherings focusing on this topic. Gradually, we became aware of the unmet need for establishing guidelines adapted to our health-care setting, which could guide physicians throughout the country on a care route that would encompass the complexity and interdisciplinary work these patients require. Many of us were trained at international referral centers for this disease in the United Kingdom, Spain, Switzerland and Portugal. We contacted the PTC Therapeutics laboratory to request logistical support (travel and one night's stay) for the meeting to finalize the manuscript, following a literature review process conducted by the discussion panel members, in which Dr. Efraín Gómez (respected cardiology professor) participated, among others.

The criteria for selecting the participants were:

- Experience in managing neuromuscular diseases
- Experience in managing patients with amyloidosis
- Publications on the topic
- Knowledge of and interest in the disease
- Willingness to work on the project without receiving fees or compensations.

A multidisciplinary panel of experts was selected, consisting of 41 specialists in different medical fields (cardiology, pulmonology, physiatry, genetics, nephrology, ophthalmology, neurology and imaging) who were invited to participate in the meeting. This list was attached to the submission, in the publication credits.

Having previously collaborated with the Integralis firm in the consensus on heart failure (in which the cardiologists of this group participated), we decided to approach them again for medical writing services so that PTC Therapeutics could stay removed from any activity related to drafting the meeting report and the article to be submitted for publication, a cost which the laboratory also decided to assume. With Integralis on the team, once the available evidence was reviewed by the specialists mentioned above, we met to draft the proposed recommendations for each of the areas. The relevant bibliography of the disease was sent to all meeting participants, including clinical practice guidelines. At the on-site meeting, the synopsis was presented by specialists in the fields of knowledge. The medical writer certified that the work was conducted independently of PTC Therapeutics.

Subsequently, the recommendations drafted for each specialty were presented and submitted to a vote. The Delphi methodology was used, with a cut-off point of

75% favorability. If this degree of favorability was not reached, the arguments in favor and against were presented by the panel and the recommendation was restructured according to the discussion. This revised recommendation was then submitted to a second vote. Once the recommendations were accepted, minor adjustments were made in their wording and content according to the participants' opinion. The main authors performed the literature review and drafted the first version of the recommendations, which was presented during the consensus meeting.

Considering that many of us have received, at some point in our practice, honorariums, scholarships, advisory boards and sponsorship of academic activities by the industry, we attach this declaration explicitly clarifying that PTC Therapeutics, in particular, never tried to influence the recommendations that appear in the article. In fact, the article revisions made by the authors after the meeting did not change the recommendations at all, since according to the established methodology, these changes required a new vote. A recording exists of the entire meeting. Therefore, we strongly reject the suggestion that this paper has been influenced by the pharmaceutical industry.

In addition, we declare, on our word of honor, that the medical writer's work was completely independent of PTC Therapeutics and only reflects information obtained during the meeting or suggested by the authors during the review process.

Regarding geneticist Diana Sánchez's current position at PTC Therapeutics, as stated by the doctor, we stipulate that when she participated in the consensus, she was not working for PTC Therapeutics, and the affiliations recorded in the article refer to her employment situation at that time.

When the article was submitted, the names of the participants who were not main authors were included in the acknowledgement section on the title page. The published article does not contain this section and, therefore, we believe that this was probably a typographical error, and therefore include the acknowledgments paragraph here.

Acknowledgements

The authors would like to thank the following physicians: Laura Peña, Christian Correa, Sergio Morales, Gina Gonzalez, Cynthia Rucinski, Efraín Gómez, Isabel Londoño, Indira Faquira, Harry Pachajoa, José Pinto Quiroz, María Paola Torres, Tatiana Bernal, Carolina Rivera, Tatiana Gómez, Julio Reyes, Sandra Zuleta, Rita

Ortega, Alba Marentes, Luis Fontanilla, Jenny Vicuña, Liliana Villareal, Diana Carolina Ramos, Sandra Castellar, Dora Inés Molina, Jaime Crump, Isabel Cárdenas, Jubby Marcela Gálvez, Gustavo Márquez, Marco Alvarado and Diego Jiménez for their participation in the consensus and contributions to the discussion.

After inquiring again about industry sponsorships received, we added the following changes to the Declaration of Conflicts of Interest:

- Erika Martínez has received honorariums from Abbott, Amgen, AstraZeneca, Bayer, Boehringer Ingelheim, Pfizer, PTC Therapeutics, Menarini, Merck S&D, Sanofi, Servier, MSD, Bristol, Novartis and Roche.
- Gina González Robledo has participated in conferences and advisory boards for Abbott/St. Jude, AstraZeneca, Bayer (Colombia and Perú), Boehringer Ingelheim, CSL Vifor International, Farma Colombia and Ecuador, Novartis, Pfizer (Colombia, Honduras, Costa Rica), PTC Therapeutics, Menarini, Sanofi, Servier and Temis Lostalo Argentina, and has received a scholarship prize from the Asociación Colombiana de Medicina Interna with Vifor International for one year of training in heart failure and transplantation in Spain. The remaining authors have nothing to declare besides PTC Therapeutics's logistical support for the meeting.