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27.09.2025 **Date**

RE: Application for the Research Award Tinnitus & Hearing 2025

Dear Professor Dr. Birgit Mazurek,

I am pleased to submit the study titled "Is *CABP2*-associated hearing loss (DFNB93) a gene therapy target? Preclinical progress and a patient registry" for consideration for the Research Award Tinnitus & Hearing 2025.

This work supports the mission of the German Tinnitus and Hearing Foundation Charité by uniting translational science, patient-centered care, and innovative infrastructure to advance therapies for hearing disorders. The *CABP2* Registry represents one of the first dedicated international efforts to establish a research-ready cohort for a rare genetic form of hearing loss that is exceptionally well suited for gene therapy. By creating a GDPR-compliant, dynamic platform that simultaneously collects robust clinical and genetic data and empowers families to connect and contribute, the registry bridges laboratory discoveries with real-world patient engagement.

The registry builds directly on my earlier experience establishing the Otoferlin Registry, which is now actively supporting recruitment and natural history studies for eventual gene therapy trials. With *CABP2*, we extend this framework to another highly promising target, ensuring that patients are not left behind as therapeutic opportunities expand. This work highlights how academic initiatives can fill critical gaps where commercial interest is limited, while also providing a model that can be replicated across other forms of hereditary hearing loss.

In line with the Foundation's goals, this project not only accelerates translational research but also fosters community, inclusivity, and patient empowerment. Families who once faced their diagnosis in isolation can now participate in research, shape clinical trial design, and access opportunities that

would otherwise remain out of reach. Recognizing this work with the Research Award Tinnitus & Hearing 2025 would help amplify the vital message that registries are indispensable for bridging discovery and patient care, ensuring Germany remains at the forefront of auditory research and clinical innovation.

Thank you for considering this application. I would be honored if this contribution could be recognized as part of the Foundation's mission to advance hearing research and improve the lives of individuals affected by hearing disorders.

Sincerely yours,

Dr. Barbara Vona

Enclosures:

- Short description of the submitted scientific work and significance
- Curriculum vitae with description of academic career
- List of publications
- Published work "Is CABP2-associated hearing loss (DFNB93) a gene therapy target? Preclinical progress and a patient registry"
- Confirmation that the work has not been submitted for any other prize with assurance that all co-authors agree to submission for the research award