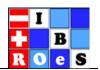
#### Wiener Biometrische Sektion der Internationalen Biometrischen Gesellschaft Region Österreich – Schweiz



## Please join the Biometric Colloquium

### **GEORG ZIMMERMANN**

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# LONGITUDINAL DATA ANALYSIS IN (PRE-)CLINICAL RESEARCH ON RARE DISEASES

### July 2<sup>nd</sup>, 2025 at 3:00 pm

Seminarraum Center for Medical Data Science (previously CeMSIIS), Spitalgasse 23, Room 88.03.513 Medical University of Vienna, 1090 Wien

Host: Franz König

### **Abstract:**

In research on rare diseases, the key outcome variables are often assessed longitudinally: Examples include tumor volumes in preclinical animal experiments, seizure counts, sizes of lesions, or patient-reported outcomes in clinical trials. Several standard statistical methods for such longitudinal settings are known and have been widely used since decades; yet, data analysis in rare diseases comes with several challenges – most prominently, (very) small sample sizes – and therefore, the question arises which statistical analysis method out of the plethora of available options should be used. In this talk, some of the specific challenges for longitudinal data analysis in rare diseases and the corresponding proposed solutions and answers will be presented. Furthermore, some open questions and potentially interesting topics for further research will be discussed.