Section 2: Executive Summary

Brainstem gliomas account for up to 20% of all CNS tumors in children with a median age at presentation of 6-7 years.[1] Diffuse intrinsic pontine gliomas (DIPG) comprise 80% of all brainstem gliomas. In North America and Europe, approximately 300 children develop brainstem gliomas per year.[2] Prognosis for patients with DIPGs remains dismal with a median survival of less than 1 year. Although radiotherapy does improve neurological function and survival by 2-3 months, no effective chemotherapeutic regimens are currently available.[1,2] Achieving cure for all children with DIPG remains a major goal of pediatric neuro-oncology. In this application, **we propose the establishment of an International Diffuse Intrinsic Pontine Glioma Registry to provide a comprehensive database of clinical, radiologic and pathologic data linked to a bioinformatics repository of molecular data of patients.** The specific aims include: a) To recruit patients diagnosed with DIPG in the International DIPG Registry; b) To provide a repository of clinical and demographic, radiological, pathologic data for patients with DIPG enrolled on the registry and maintain annual follow-up on all cases; c) To develop a bioinformatics repository of existing molecular data on DIPGs that can be linked to patient information in the registry; d) To establish collaborations among investigators for hypothesis-driven research studies through the registry that will ultimately lead to better classification and more effective treatment of patients with DIPG. The first two projects proposed for specific aim 4 are a comprehensive study of long-term DIPG survivors based on data (clinical, radiographic, molecular and pathologic) captured in the registry, as well as an epidemiological study to assess the incidence patterns of DIPG in North America. **Our long-term goal is to establish and maintain a highly collaborative, international, hypothesis-driven research infrastructure that can support a wide spectrum of interdisciplinary and translational projects in DIPGs for all investigators**. The data collected form a research continuum from basic biology to clinical practice that will ultimately address our primary goals of a) understanding the biology of DIPGs, b) developing more effective therapies and c) developing new approaches to diagnosis, response assessment and multidisciplinary treatment and follow-up that will improve patient outcome.