ABSTRACT

Atypical teratoid rhabdoid tumor (ATRT) is an aggressive pediatric CNS tumor yet lacking standardized therapies while current management standards are not of significant clinical benefit. With the opportunity of accessing the Childhood Cancer Model Atlas (CCMA)'s vast pediatric cancer resources, high-throughput drug screening (HTS) was carried out on the CCMA's ATRT cohort against the novel Australian Library of Paediatric Anti-Cancer Agents (ALPACA) to identify novel therapeutic strategies for ATRT. Drug hits primarily reveal the vulnerability of ATRT towards cell cycle inhibition, but also potential ATRT-selective and ATRT subgroup-selective therapies. Given the less-quantitative nature of HTS, these findings are a preliminary insight on therapeutic opportunities for ATRT to be further evaluated in future studies.

Keywords: atypical teratoid rhabdoid tumor, high-throughput drug screening, novel therapy, cell cycle inhibitor, Idebenone, AZD1208