OBJECTIVES

CB-derived cellular therapies are being evaluated as a potential treatment for a growing number of neurological conditions, including cerebral palsy (CP) and other static encephalopathies (CP/SE). LUSTRE™ (Umbilical Stem cell monitoring and Treatment REsearch) is a registry enrolling children with CP/SE and other conditions whose families have privately stored CB. LUSTRE™ provides insight into the types, causes, and severity of conditions affecting patients with stored CB, regardless of whether these patients receive CB infusions.

METHODS

LUSTRE™ is open to all families who have stored CB in a large, U.S.-based private CB bank. It collects data in three distinct phases (Figure 1). I) The Surveillance Phase collects data on the prevalence of medical conditions among families storing CB. II) The Monitoring Phase collects detailed data on children with target conditions identified in Phase I, including disease severity, treatment history, demographic and quality of life information. Elements of these data are aggregated and displayed to participants in a secure online database. III) The Infusion Phases are open to eligible children whose CB is stored (the “donor”) and their biological siblings who do not receive CB infusions. The Infusion Phases collect additional clinical details about subjects who receive CB infusions. Where appropriate, referrals to clinical trials are made through the Surveillance and Monitoring Phases. All study data collection occurs online and is predominantly patient-reported. The Monitoring and Infusion Phases are open to eligible children whose CB is stored (the “donor”) and their biological siblings who do not receive CB infusions.

RESULTS

Surveillance Phase: Since October 2013, 117,834 families have completed a surveillance survey. 1,059 (0.9%) indicated CP/SE diagnoses, either in a child with stored CB or in their sibling. Monitoring Phase: From a pool of 462 eligible donor children, we recruited 185 participants (40.0%), 37 of whom had received a previous CB infusion. Of these 185 participants, 56% were male with an average age at enrollment of 5.4 years (Table 1). Greater than 85% of study subjects were White. Non-Response Rate of parents reporting receiving at least a four-year college degree, and 47.9% reported an annual household income of $100,000 or greater. 40.0% of our cohort were diagnosed with CP/SE or other SE. Among parents, 40% reported having a CB of specific CP type (spastic, hypotonic, or dyskinetic), and 30% indicated their child was born outside of the U.S. 50.0% of the children diagnosed with CP reported bilateral body involvement (18.0% diplegia and 32.0% hemiplegia), and 40.0% reported unbalanced (hemiplegic) body involvement. Infusion Phase: 46 children in the CP/SE cohort have received a CB infusion (11 completed a CB stem cell infusion). These results suggest that families with a child with severe CP are more likely to choose a CB stem cell infusion based on the few children (n=11) for whom CB infusion is based on the few children (n=11) for whom CB infusions to be performed. Of these patients, 91.0% had received a diagnosis of CP/SE within the last two years prior to infusion. These results suggest that families with a child with severe CP are more likely to choose a CB stem cell infusion based on the few children (n=11) for whom CB infusion is performed.