

## New provincial initiatives for childhood disabilities: the imperative for research

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**H**igh-profile initiatives aimed at improving outcomes for children with developmentally disabling conditions are long overdue. For example, to ensure early identification and intervention for infants with congenital hearing loss, Ontario is poised to implement universal hearing screening for newborns,<sup>1</sup> Alberta has announced funding for a substantial pilot project for the same type of screening,<sup>2</sup> and Health Canada has established the Canadian Working Group on Childhood Hearing.<sup>3</sup> For young children with autism, Alberta and Ontario have launched intensive province-wide intervention programs based on the principles of applied behavioural analysis, and, after a recent provincial supreme court decision declaring that applied behavioural analysis is “medically necessary treatment,” British Columbia may soon follow suit. However, the standards of evidence that have led to these changes in public policy create a quandary for physicians and others who consider themselves advocates for patients, yet who also value the importance of evidence-based health care: how to respond constructively to government-funded programs that address stakeholders’ needs and have the potential to help patients but that have not been adequately studied.

Universal newborn hearing screening, the first example mentioned above, is endorsed by a broad consensus of professional opinion,<sup>4</sup> but it has never been subjected to a clinical trial of the kind demanded by contemporary standards for evidence-based health care.<sup>5</sup> The traditional criteria for implementing screening include sensitive, accurate and acceptable tests; an agreed-upon policy determining whom to treat; established treatment that is accessible and effective; and solid evidence that early intervention improves outcomes.<sup>6</sup> Progress in audiometric technology has made universal testing of newborns’ hearing feasible, and studies have shown that for children in whom congenital hearing loss was identified before the age of 6 to 12 months, speech and language skills were better at 3 to 5 years of age than those of children in whom hearing loss was discovered later.<sup>7,8</sup> However, longer term developmental gains in real-world populations undergoing universal screening have not yet been documented. The place of sign language and cochlear implants in the habilitation of deaf children remains controversial, which has prevented consensus on intervention. The detection, through universal screening, of children with mild, unilateral hearing loss will challenge parents and professionals to ensure specific benefits for a group whose

needs are poorly understood, while avoiding the negative effects of “labelling.” Little is known about other possible harms of universal hearing screening, including the impact of false positives on early parent–infant relationships and the likelihood that infants will be subjected to inappropriate otological procedures or to otological surgery that is not associated with benefit but carries risk of harm.<sup>9</sup>

Two particular concerns with universal hearing screening of newborns are its failure to identify a potentially substantial group of infants with latent or progressive forms of hearing loss due to prenatal cytomegalovirus infection<sup>10</sup> and the lack of access to specialized services for children with hearing loss who live outside urban centres. Furthermore, data are lacking to compare the benefits and costs (including the opportunity cost) of universal screening with the benefits and costs of alternative early-identification strategies, such as screening of all infants who meet specific high-risk criteria, combined with educational efforts to sensitize parents and health care providers to early identification of hearing loss.<sup>11</sup>

With regard to the example of autism, available evidence suggests that early intervention improves outcomes for affected children.<sup>12</sup> Intensive intervention with behavioural teaching methods has been studied in single-subject trials and in 6 demonstration projects using before-and-after (uncontrolled) or cohort designs. Most often cited is the work of Ivor Lovaas,<sup>13</sup> who reported substantial gains in cognitive scores and overall functional status for 19 children who received 40 h/week of a one-on-one intervention based on applied behavioural analysis; children receiving minimal intervention did not experience such gains. This work has not been fully replicated, and estimation of the true effect size was obscured by subject selection biases, nonrandomized assignment and problems with how the outcomes were evaluated. These promising but methodologically limited findings have led to the current dilemma of how to balance the immediate need for effective intervention for autistic children against the need for clear information on how outcomes are related to particular treatment elements and program intensity.

Effective treatment programs for autism share a number of common elements, including age at entry (preschool years) and intensity (at least 20 h/week),<sup>12</sup> but these variables have never been systematically varied to determine their relation to outcomes. Moreover, programs of similar intensity have never been directly compared, so

many questions remain regarding optimum setting, curriculum and teaching approach.<sup>12</sup> In addition, little is known about what characteristics of the child predict treatment response or what intensity, duration and treatment model are most effective for particular children. A final and contentious issue is the specification of entrance criteria for new provincial programs for autism. The Ontario program is designed to target children at the “severe” end of the autism spectrum (a term that has not yet been operationally defined), but there is no evidence that children with severe symptoms of autism are more likely to respond than children with milder symptoms. In fact, the first reported randomized trial of applied behavioural analysis found that children on the milder end of the spectrum (i.e., those who did not meet all of the criteria for autism) might actually have benefited more than children with clearly diagnosed autism.<sup>14</sup>

“Complex interventions to improve health” can and should be evaluated.<sup>15</sup> Ideally, such evaluation would guide decisions about program implementation. For example, with provincial government funding, investigators in Quebec have just completed a clinical trial of hyperbaric oxygen therapy for children with cerebral palsy.<sup>16</sup> The exemplary methodology of this timely study, which found that hyperbaric oxygen therapy did not improve function, has yielded sound evidence that may ultimately shape policy decisions. However, even after program start-up, research is needed to influence further decisions about implementation and to evaluate program quality so that operating funds are used in the most rational, responsible and fair way. The key questions in the 2 examples that have been described in detail, universal newborn hearing screening and autism intervention, involve identifying the specific entry and treatment parameters that will maximize benefit to particular subgroups of participants while minimizing possible harms. Carefully designed cohort and experimental studies with adequate longitudinal follow-up can answer these questions and increase our knowledge of the natural history of children with congenital hearing loss and autism.

The principles we have discussed here go beyond these specific programs designed for children with disabilities. We recommend that federal and provincial governments include research as an integral part of planning and implementing *all* new major programs. Traditional granting agencies offer some support for such research, but, given the significance of the policy decisions that will be taken, additional resources will usually be needed. We hope that governments and researchers will rise to the challenge of this historic opportunity to ensure that children and families benefit from both existing knowledge on interventions and future efforts to fill the outstanding gaps in evidence.

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