Flash-lamp pulsed-dye laser treatment of port-wine stains in childhood. A case of technology assessment

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Summary
Before the availability of the flash-lamp pulsed-dye laser (FPDL) patients with vascular birthmarks were treated with lasers that had a significant risk of scarring and were therefore not selected for the treatment of children. With the introduction of the flash-lamp pumped pulsed-dye laser in 1985 it was assumed that port-wine stains in childhood could be treated safely, effectively and probably also more efficiently. In this thesis, the introduction and dissemination of the flash-lamp pulsed-dye laser for the treatment of port-wine stains in childhood is evaluated.

In Chapter 1 the results of a prospective study, 'Treatment of port-wine stains by laser, the younger the better', regarding the effectiveness of the flash-lamp pumped pulsed-dye laser in relation to age are reported. We studied hundred consecutive patients, divided into four age groups (0 to 5, 6 to 11, 12 to 17 and 18 to 31 years). The outcome measure was clearing of the port-wine stain as measured with a colorimeter. The average reduction in the difference in color between the port-wine stain and the contralateral normal skin was 40 percent after an average of 5 treatments of the entire port-wine stain. Differences between age groups in the average reduction in color differences were not significant (p=0.26). We found no evidence that treatment of port-wine stains with the flash-lamp pulsed-dye laser in early childhood is more effective than treatment at a later age.

The disfigurement of a visible port-wine stain can cause a negative impact on emotional and psychological well being of the patient. In Chapter 2 the impact of a facial port-wine stain on behavioural problems in children and on health related quality of life in adolescents and adults, applying for laser treatment, is evaluated. A survey by questionnaire of a hundred consecutive port-wine stain patients, without former treatment of their port-wine stain was performed. Two standard general instruments and a more specific port-wine stain questionnaire were used. Childrens’ (4-12 years) parents received the Child Behaviour Checklist (CBCL). Adolescents and adults (13-31 years) received a short version of the RAND Health Insurance Questionnaire from the Medical Outcome Study (MOS-24). A specific PWS questionnaire was sent to both age groups. Observed problem behaviour in children was compared to a Dutch reference group, from children with constipation and from an other group of children with a port-wine stain. The results of the MOS-24 were compared with those of a reference population without chronic illness. Childrens’ parents reported no clinically significant problem behaviour. Adolescents and adults showed low scores for mental health and self-perceived health. They reported less vitality/energy than was expected in this
age group. Compared to children, adolescents and adults experienced more negative consequences of their facial port-wine stain in social contacts (p<0.01).

It is believed that port-wine stains in children are smaller and thinner, therefore treatment at an early age would not only be desirable but also more efficient. **Chapter 3** links the timing of treatment to the societal costs. The economic efficiency of early treatment of port-wine stains versus treatment at an advanced age has been evaluated. A prospective comparative study was set up within four age groups (0 to 5, 6 to 11, 12 to 17 and 18 to 31 years) of patients with a facial port-wine stain. We included 91 patients. All patients received 5 treatments of the entire port-wine stain. The average direct medical costs per patient did not differ significantly between age groups (p=0.80). In all age groups costs of treatment increased proportionally with the absolute size of the port-wine stain. We concluded that, despite the smaller surface area of the port-wine stain, the number of visits, pulses needed and treatment costs were not lower in children. These findings were influenced by the use of general anesthesia in children. Treatment at early ages appeared to be more difficult as expected and less efficient compared to adolescents and adults.

**Chapter 4** presents an outline of the main problems and risks associated with the port-wine stain treatment of children. A study was performed in 43 consecutive young patients (4-12 years) with a facial port-wine stain. Two separate evaluation questionnaires were distributed, completed by the parents of the patients. Children went through a mean treatment period of 2.5 years. General anesthesia had to be used during treatment in 13 children (32%). Parents reported an extra time-investment in the week following treatment to give extra attention to the child and to take care of the treated port-wine stain. Most children (95%) missed two days of school for each visit. Only a small number of parents regarded the laser treatment as not painful. Fifty percent felt the laser treatment to be a burden for their child. Eighty percent reported that their child disliked the idea of laser treatment. Purpera after treatment was experienced as a stigma. Overall, parents (85%) felt that it took a long time to gain optimal clearance. The decision whether or not to start port-wine stain treatment in childhood should not be based on clinical outcome only but also on a considerate weighing of the additional consequences: pain management, the requirement of multiple treatments, discomfort, anxiety and the motivation of the child.
Summary

In **Chapter 5** we prospectively studied the assessment of the outcome of therapy during childhood. Fifty-two parents of patients (aged 0 to 13) were asked to evaluate their expectations of flash-lamp pulsed-dye laser therapy and outcome of treatment of their children. During laser treatment a parent-report questionnaire was developed, covering the following topics: treatment expectations, adequacy of the received information on treatment, global satisfaction with present appearance, and an appraisal of the achieved results in economic terms. At the time of evaluation children went through a mean treatment period of 2.4 years. At the beginning of treatment sixty percent of the parents reported that they would accept a negative outcome. Most parents were satisfied with the received information prior to treatment. Although fifty percent of the parents would have preferred to receive more information about the duration of treatment. The majority of the children's parents (90%) felt that the port-wine stain of their child has become less striking as a result of therapy. Parents were also asked to evaluate satisfaction with treatment using the willingness to pay method (anchored from 0 to 1500 Dutch Guilders). Parents of young children (aged 0 to 5) reported higher amounts compared to the parents of older children, aged 6 to 13 years, differences were not statistically significant. Parents of patients who had received a high number of total treatments of the entire port-wine stain expressed less satisfaction with outcome. Although clearing of port-wine stains in children is partial and treatment of children took considerably more time than anticipated, the conclusion is that parents of patients were satisfied with the overall results.

**Chapter 6** describes the introduction and dissemination of the flash-lamp pulsed-dye laser into port-wine stain treatment of children and its use between 1994 and 1999 in the Netherlands. A prospective survey of 11 centers that had gained experience with the treatment of port-wine stains and the use of laser equipment was conducted. In 1994 semi-structured interviews were recorded in all centers and in 1999 an evaluative telephone survey was performed. It appeared that the flash-lamp pulsed-dye laser has become the treatment of choice for port-wine stains in childhood. Academic hospitals and teaching hospitals were fast adopters of this new treatment modality. Within the study period the technical refinement of laser treatment dominated all evolution. Inappropriate and unskilled use is at present one of the greatest problems with laser applications. Treatment procedures are still not standardized. We conclude that between 1994 and 1999 laser treatment characteristics and procedures have been established by consensus, rather than by adequate explicit guidelines.
Chapter 7 discusses the introduction and the use of the flash-lamp pulsed-dye laser for the treatment of port-wine stains, from the perspective of Health Care Technology Assessment. Using the Technology Assessment Iterative Loop (TAIL), it was examined whether sufficient evidence from evaluations has been present to support the introduction of the flash-lamp pulsed-dye laser at an early age. Such an assessment requires an interdisciplinary approach focusing on aspects of safety, efficacy, effectiveness, health-related quality of life, societal costs, as well as the ethical issues of treatment. Assessment of the flash-lamp pulsed-dye laser in port-wine stain treatment of children did not follow the iterative model of medical innovation and evaluation. Nearly all assessments of effectiveness focused on laser applications that were already in clinical use. Systematic information evaluating the use of the flash-lamp pulsed-dye laser at any age was not available at any practical stage. Efficacy and effectiveness have been the major concern in most evaluative studies. Societal costs and ethical aspects of treating children were seldom included. In conclusion it can be stated that the introduction and diffusion of the use of the flash-lamp pulsed-dye laser in the treatment of PWS in childhood was uncontrolled and the field was not prepared to use this technique appropriately.