Editorial

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Childhood craniopharyngioma – current status and recent perspectives in diagnostics and treatment

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The Third International Multidisciplinary Postgraduate Course on Childhood Craniopharyngioma 2014, at Bad Zwischenahn, Germany, was attended by 60 participants from 21 countries. The present special issue of the Journal of Pediatric Endocrinology and Metabolism contains selected scientific contributions summarizing the current status on diagnostics and treatment of patients with childhood-onset craniopharyngioma.

Adamantinomatous craniopharyngiomas are histologically benign but clinically aggressive epithelial tumors of the sellar region that are associated with high morbidity and occasional mortality. Research from the last 3 years has provided important insights into the molecular and cellular pathogenesis of these tumors. It has become established that mutations in CTNNB1 (encoding β-catenin), leading to the over-activation of the WNT pathway, underlie the molecular etiology of human adamantinomatous craniopharyngiomas. Martinez-Barbera and Buslei (1) report on a novel and unexpected role for pituitary stem cells, which is fundamentally distinct from the cancer stem cell paradigm. The study of these benign tumors could reveal important insights into general mechanisms underlying the initial steps of tumorigenesis and facilitate novel tools to improve management of the patients.

Current concepts in the neurosurgical treatment of craniopharyngioma remain controversial. Flitsch et al. (2) state that surgery remains the first treatment option in pediatric craniopharyngiomas. However, with modern techniques and strategies, the functional outcome has been improved at the expense of incomplete resections. Hypothalamic damage should be of main concern prior to and during any surgical procedure, as currently no sufficient therapy of hypothalamic syndrome is available. Radiotherapy for residual tumor control is an important adjunct. A life-long endocrinological and neuroradiological follow-up is recommended.

Due to tumor and treatment-related sequelae the long-term morbidity is substantial with hypopituitarism, increased cardiovascular risk, hypothalamic damage, visual and neurological deficits, reduced bone health, and reduced quality of life and cognitive function. Erfurth (3) reports on a 3–19 fold higher cardiovascular mortality in comparison to the general population in adult-onset craniopharyngiomas.

Based on findings of reduced energy expenditure and frequent neuropsychological impairments, the impact of eating behavior and the rate of eating disorders associated with hypothalamic obesity in childhood craniopharyngioma are unknown. For the first time, Hoffmann et al. (4) analyzed the eating behavior in a large cohort of craniopharyngioma patients and reported on the pathological findings associated with severe obesity. However, eating behavior and eating disorders of craniopharyngioma patients were similar when compared to age- and body mass index-matched normal controls.

Treatment options for hypothalamic obesity in craniopharyngioma are limited. Kalina et al. (5) report on promising short-term effects of a combination therapy with metformin and fenofibrate. van Santen et al. (6) presents a case treated with T3 mono-therapy, not supporting a previous report on the beneficial effects of T3 mono-therapy on hypothalamic obesity.

Not only multidisciplinary decisions on treatment strategies (7), but also interdisciplinary communication of strategies with patients has important benefits. Shared decision-making has been termed the “pinnacle of patient-centered care”. Nemergut and Townsend (8) report on their experiences as parents of a daughter with childhood craniopharyngioma. The authors come to a conclusion that emphasizing protocols that foster a team-based approach to communication can improve both the efficiency and quality of medical decisions, while best capturing the new spirit of patient-directed care.

Risk-adapted surgical strategies at initial diagnosis of childhood craniopharyngioma should aim at a maximal degree of resection, keenly focused on respecting the integrity of optical and hypothalamic structures to prevent severe sequelae and therein minimize consequences that
could have a negative impact on patient’s quality of life. In case of hypothalamic involvement, hypothalamussparing surgical strategies are recommended in order to prevent hypothalamic damage and associated severe sequelae. Local irradiation of residual tumor is efficient in preventing tumor progression.

Because initial hypothalamic tumor involvement, especially of posterior hypothalamic structures, has an a priori effect on the clinical course, childhood craniopharyngioma should be recognized as a chronic disease requiring constant monitoring of the consequences and medical resources for treatment, in order to provide not only optimal quality of life for patients, but also to garner additional information with the intent of minimizing what at present are severe consequences of both the disease and its treatment. An intensification of international collaboration in basic and clinical research is necessary to improve treatment and outcome of patients with rare diseases such as childhood-onset craniopharyngioma.

References

8. Nemergut DR, Townsend AR. The importance of interdisciplinary communication with patients about complex, chronic illnesses: our experiences as parents of a child with a craniopharyngioma. J Pediatr Endocrinol Metab 2015; in press.

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