BÖLÜM 10

ÇOCUK NÖROLOJİ HASTALARINDA KEMİK İLİĞİ NAKLİ VE İLERİ HEMATOLOJİK TEDAVİ SEÇENEKLERİ

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GİRİŞ

Kemik iliği nakli, benign ve malign hematolojik hastalıklar, immün yetersizlik, doğuştan metabolik bozukluklar gibi çocukluk çağında rastlanan pek çok hastalığın tedavisinde tedavi protokollerinin bir parçası ve/veya tek iyileştirici tedavi seçeneği olarak yer almaktadır. Kök hücre kaynağı olarak kemik iliği dışında periferik kan kök hücre, kordon kanı gibi diğer kök hücre kaynaklarının da kullanıma girmesi ile 'kemik iliği nakli' yerine 'hematopoetik kök hücre nakli' (HKHN) terimi kullanılmaya başlanmıştır.[1]

Otolog ve allojenik HKHN bazı nörolojik bozuklukların tedavisinde yer almaktadır. Multipl Skleroz gibi (MS) immun aracılı hastalıklarda otolog HKHN ve metabolik yollarda yer alan enzimlerdeki konjenital eksikliklerle karakterize durumlarda ise allojenik HKHN tercih edilmektedir.[2]

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Pediatrik beyin tümörlerinin tedavisinde kontrol noktası blokajının kullanımı da aktif bir araştırma alanı olmuştur. Programlanmış hücre ölüm proteini 1 (PD1) ve sitotoksik T lenfosit ilişkili protein 4 (CTLA4) gibi kontrol noktası molekülleri, T hücrelerinin yüzeyinde bulunan ve bağışıklık tepkisini düzenlemeye yardımcı olan proteinlerdir. Kendi kendine toleransın geliştirilmesinde yer alırlar, ancak kanserler bu yolu tümöre özgü bağışıklık tepkisini azaltmak veya kaçmak için kullanabilir.[26] Tekrarlayan pediatrik beyin tümörleri için bir anti-PD1 molekülü olan nivolumab ve anti-CTLA4 olan ipilimumab ile ilgili açık bir çalışma mevcuttur (NCT03130959).

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